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**UNITED STATES
SECURITIES AND EXCHANGE COMMISSION**
Washington, D.C. 20549

Form 10-K

(Mark one)

ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934

For the fiscal year ended December 31, 2013

or

TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934

For the transition period from _____ to _____

Commission file number 001-36020

Onconova Therapeutics, Inc.

(Exact name of registrant as specified in its charter)

Delaware (State or other jurisdiction of incorporation or organization)	22-3627252 (I.R.S. Employer Identification No.)
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375 Pheasant Run, Newtown, PA (Address of principal executive offices)	18940 (Zip Code)
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(267) 759-3680
(Registrant's telephone number, including area code)

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

<u>Title of each class</u>	<u>Name of each exchange on which registered</u>
Common Stock, par value \$.01 per share	NASDAQ Global Market

Securities registered pursuant to Section 12(g) of the Act: **None**

Indicate by check mark if the registrant is a well-known 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 (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes No

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K is not contained herein, and will not be contained, to the best of registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K.

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, or a smaller reporting company. See the definitions of "large accelerated filer," "accelerated filer" and "smaller reporting company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer

Accelerated filer

Non-accelerated filer

Smaller reporting company

(Do not check if a
smaller reporting company)

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

As of June 28, 2013 (the last business day of the registrant's most recently completed second fiscal quarter), the registrant's common stock was not listed on any exchange or over-the-counter market. The registrant's common stock began trading on the NASDAQ Global Market on July 25, 2013. As of February 28, 2014, the aggregate market value of the registrant's voting stock held by non-affiliates was approximately \$107.3 million based on the number of shares held by non-affiliates as of February 28, 2014, and the last reported sale price of the registrant's common stock on February 28, 2014.

There were 21,638,029 shares of Common Stock outstanding as of February 28, 2014.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of our Proxy Statement for the 2014 Annual Meeting of Stockholders, to be filed within 120 days of December 31, 2013, are incorporated by reference in Part III. Such Proxy Statement, except for the parts therein which have been specifically incorporated by reference, shall not be deemed "filed" for the purposes of this Annual Report on Form 10-K.

ONCONOVA THERAPEUTICS, INC.

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SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS AND INDUSTRY DATA

This Annual Report on Form 10-K ("Annual Report") includes forward-looking statements. We may, in some cases, use terms such as "believes," "estimates," "anticipates," "expects," "plans," "intends," "may," "could," "might," "will," "should," "approximately" or other words that convey uncertainty of future events or outcomes to identify these forward-looking statements. Forward-looking statements appear in a number of places throughout this Annual Report and include statements regarding our intentions, beliefs, projections, outlook, analyses or current expectations concerning, among other things, our ongoing and planned preclinical development and clinical trials, the timing of and our ability to make regulatory filings and obtain and maintain regulatory approvals for our product candidates, our intellectual property position, the degree of clinical utility of our products, particularly in specific patient populations, our ability to develop commercial functions, expectations regarding clinical trial data, our results of operations, cash needs, financial condition, liquidity, prospects, growth and strategies, the industry in which we operate and the trends that may affect the industry or us.

By their nature, forward-looking statements involve risks and uncertainties because they relate to events, competitive dynamics and industry change, and depend on the economic circumstances that may or may not occur in the future or may occur on longer or shorter timelines than anticipated. Although we believe that we have a reasonable basis for each forward-looking statement contained in this Annual Report, we caution you that forward-looking statements are not guarantees of future performance and that our actual results of operations, financial condition and liquidity, and the development of the industry in which we operate may differ materially from the forward-looking statements contained in this Annual Report. In addition, even if our results of operations, financial condition and liquidity, and events in the industry in which we operate are consistent with the forward-looking statements contained in this Annual Report, they may not be predictive of results or developments in future periods.

Actual results could differ materially from our forward-looking statements due to a number of factors, including risks related to:

- our estimates regarding expenses, future revenues, capital requirements and needs for additional financing;
- the success and timing of our preclinical studies and clinical trials;
- the difficulties in obtaining and maintaining regulatory approval of our product candidates, and the labeling under any approval we may obtain;
- our plans and ability to develop and commercialize our product candidates;
- our failure to recruit or retain key scientific or management personnel or to retain our executive officers;
- the size and growth of the potential markets for our product candidates and our ability to serve those markets;
- regulatory developments in the United States and foreign countries;
- the rate and degree of market acceptance of any of our product candidates;
- obtaining and maintaining intellectual property protection for our product candidates and our proprietary technology;
- the successful development of our commercialization capabilities, including sales and marketing capabilities;
- recently enacted and future legislation regarding the healthcare system;
- the success of competing therapies and products that are or become available; and

- the performance of third parties, including contract research organizations, or CROs and third-party manufacturers.

Any forward-looking statements that we make in this Annual Report speak only as of the date of such statement, and we undertake no obligation to update such statements to reflect events or circumstances after the date of this Annual Report or to reflect the occurrence of unanticipated events. Comparisons of results for current and any prior periods are not intended to express any future trends or indications of future performance, unless expressed as such, and should only be viewed as historical data.

You should also read carefully the factors described in the "Risk Factors" section of this Annual Report and elsewhere to better understand the risks and uncertainties inherent in our business and underlying any forward-looking statements. As a result of these factors, we cannot assure you that the forward-looking statements in this Annual Report will prove to be accurate. Furthermore, if our forward-looking statements prove to be inaccurate, the inaccuracy may be material. In light of the significant uncertainties in these forward-looking statements, you should not regard these statements as a representation or warranty by us or any other person that we will achieve our objectives and plans in any specified timeframe, or at all.

We obtained the industry, market and competitive position data in this Annual Report from our own internal estimates and research as well as from industry and general publications and research surveys and studies conducted by third parties. Industry publications and surveys generally state that the information contained therein has been obtained from sources believed to be reliable. We believe this data is accurate in all material respects as of the date of this Annual Report.

PART I

ITEM 1. BUSINESS

Overview

We are a clinical-stage biopharmaceutical company focused on discovering and developing novel small molecule drug candidates to treat cancer. Using our proprietary chemistry platform, we have created an extensive library of targeted anti-cancer agents designed to work against specific pathways that are important to cancer cells. We believe that the drug candidates in our pipeline have the potential to be efficacious in a variety of cancers without causing harm to normal cells. Currently, we have three clinical-stage product candidates and six preclinical programs.

Rigosertib, our most advanced product candidate, is being tested in clinical studies for myelodysplastic syndromes, or MDS, and head and neck cancers. To date, we have enrolled more than 1,000 patients in rigosertib trials in hematological diseases and solid tumors. We are testing both intravenous and oral formulations of rigosertib, referred to as rigosertib IV and rigosertib Oral.

In February 2014, we reported that the Phase 3 "ONTIME" trial of rigosertib IV in patients with higher risk MDS who had progressed on, failed to respond to or relapsed after prior therapy with hypomethylating agents, or HMAs, did not meet the primary endpoint of improving overall survival compared to best supportive care, or BSC. The ONTIME trial enrolled 299 patients, including 199 patients in the rigosertib IV plus BSC arm. Median overall survival in the rigosertib IV plus BSC arm was 8.2 months compared to 5.8 months in BSC only arm. Treatment with rigosertib IV plus BSC did not demonstrate a statistically significant improvement in median overall survival when compared to BSC only (Hazard Ratio=0.86; p-value=0.27). However, a post-hoc analysis demonstrated a statistically significant increase in median overall survival in the subset of patients who had progressed on or failed to respond to previous treatment with HMAs, thus demonstrating potential activity of rigosertib in these MDS patients. In this subset of patients, the median overall survival was 8.5 months in the rigosertib IV plus BSC arm compared to 4.7 months in BSC only arm (Hazard Ratio=0.67; p-value=0.022). In this patient population, 127 patients were in the treatment arm and 57 patients were in the BSC arm. The other subset, which was comprised of patients who had relapsed after responding to previous treatment with HMAs (115 of 299 patients enrolled), did not show a statistically significant survival benefit. Preliminary safety analysis from the trial indicates that rigosertib was generally well tolerated in the study population. We will consult with regulatory authorities to determine the next steps for rigosertib IV in higher risk MDS.

A Phase 3 trial of rigosertib IV in higher risk MDS patients who have progressed on HMA therapy is now enrolling patients in U.S. and European Sites. This single-arm study is designed for 90 patients. Currently, 19 patients have been enrolled in this study.

In December 2013, we reported results from a Phase 2 trial with rigosertib Oral in transfusion-dependent lower risk MDS at the 2013 American Society of Hematology (ASH) Annual Meeting. Based on Phase 2 data presented at the 2013 ASH meeting, we consulted with the U.S. Food and Drug Administration, or FDA, regarding further development of rigosertib Oral for transfusion-dependent lower risk MDS patients. As a result of this dialogue, we have designed a pivotal Phase 3 protocol. We intend to seek a Special Protocol Assessment, or SPA, for this randomized, double-blind, placebo-controlled study in lower risk MDS patients who do not respond to erythropoiesis-stimulating agents, or ESAs, and are dependent on packed red blood cell transfusions to alleviate their anemia. Total number of patients, entry criteria, secondary endpoints, and the statistical analysis plan will be finalized during the SPA process. We project to start enrollment in the Phase 3 study in the second half of 2014 at multiple sites in the United States and Europe. We are also evaluating a prognostic tool based on genomic methylation analysis in this patient population. Currently, a validation cohort of 20 patients is being enrolled in a Phase 2 trial with the objective of assessing the value of the prognostic genomic

methylation marker for future studies. The FDA has encouraged us to discuss these results with them for potential integration of this analytical tool in the pivotal study. We expect to report on these studies and discussions in the second half of 2014. We have obtained rights to intellectual property covering this method of prognosis from Columbia University.

We are also evaluating rigosertib Oral in combination with azacitidine in a Phase 1/2 trial for first-line treatment of MDS. A Phase 1/2 study is now underway to evaluate the potential synergistic activity of rigosertib and azacitidine. In this study, the full indicated dose of azacitidine is given in combination with escalating doses of rigosertib Oral in successive cohorts. Rigosertib doses of 140-560 mg given two times daily (BID) will be tested. After the dose-finding segment of the trial is completed, the second part of the trial will evaluate the selected dose in additional patients. A total of up to 40 patients are planned to be enrolled in this study. This study is now running in U.S. sites and has enrolled seven patients. We expect to provide updates on safety, tolerability and preliminary evidence of anti-tumor activity in the second half of 2014.

We are also testing rigosertib in solid tumors. A Phase 2 clinical trial is underway in head and neck cancer.

In December 2013, following a pre-planned interim analysis, we announced the discontinuation of a Phase 3 study of rigosertib in combination with gemcitabine in first-line metastatic pancreatic cancer.

Rigosertib has been granted orphan drug status for MDS in the United States, Europe, and Japan. Baxter Healthcare SA, or Baxter, a subsidiary of Baxter International Inc., has commercialization rights for rigosertib in Europe and Symbio Pharmaceuticals Limited, or Symbio, has commercialization rights in Japan and Korea. We retain development and commercialization rights to rigosertib in all other territories, including in the United States.

Rigosertib inhibits two important cellular signaling pathways involving phosphoinositide 3-kinase, or PI3K, and polo-like kinase, or PLK, through a novel mechanism of action. PI3K signaling promotes the growth and survival of cells under stressful conditions, such as under low oxygen levels that are often found in tumors. By inhibiting the PI3K pathway in cancer cells, rigosertib promotes tumor cell apoptosis, or programmed cell death. The PLK pathway has a critical role in maintaining proper chromosome organization and sorting during cell division. By modulating the PLK pathway, rigosertib stops cancer cells at late stages of the cell division cycle, which leads to chromosome disorganization and death in these cells. In normal cells, rigosertib reversibly pauses progression of the cell cycle in the early stages, without causing harm or death to these cells. We recently presented new data on the mode of action of rigosertib. These data indicate that rigosertib binds to the Ras Binding Domain (RBD) found in many signaling proteins, including Raf kinases and PI3K. This specific binding disrupts protein-protein interactions necessary for signal transduction in the cell. This signal transduction inhibitory activity suggests novel ways in which cellular pathways could be interrupted by rigosertib and informs the design of future translational studies.

Onconova is developing rigosertib IV and rigosertib Oral for the following unmet medical needs:

- *Rigosertib IV as a second-line therapy in higher risk MDS:* In February 2014, we reported that the Phase 3 ONTIME trial of rigosertib IV in patients with higher risk MDS who had progressed on, failed to respond to or relapsed after prior therapy with HMAs did not meet the primary endpoint of improving overall survival compared to BSC. However, a post-hoc analysis demonstrated a statistically significant increase in median overall survival in the subset of patients who had progressed on or failed to respond to previous treatment with HMAs, thus demonstrating potential activity of rigosertib in these MDS patients. Preliminary safety analysis from the trial indicates that rigosertib was generally well tolerated in the study population. We will consult with regulatory authorities to determine the next steps for rigosertib IV in higher risk MDS.

MDS is a group of blood disorders caused by altered bone marrow function. We believe that there is a significant medical need for new therapies to treat MDS patients who have failed or cannot tolerate treatment with the hypomethylating drugs azacitidine (Vidaza®) or decitabine (Dacogen®), which represent the current standard of care for higher risk MDS patients. To our knowledge, there are no other current Phase 3 trials in this patient population. A provider of marketing analytics and data to the biopharmaceutical industry has estimated that, for 2011 in the United States, the diagnosed incidence of MDS was approximately 15,600 and the prevalence of MDS was approximately 52,000. According to the same marketing analytics firm, approximately 23% of MDS patients are estimated to fall into the categories of MDS characterized as higher risk.

- *Rigosertib Oral as a first-line therapy in transfusion-dependent lower risk MDS:* We reported results from a Phase 2 trial with rigosertib Oral in transfusion-dependent lower risk MDS at the 2013 ASH Annual Meeting. Overall, transfusion independence was observed in 39% (14 of 36) of patients receiving at least eight weeks of the intermittent dosing schedule of rigosertib Oral. A combined response rate of 53% (according to International Working Group criteria) was also observed in these patients. Following recent consultations with the FDA, we expect to begin a Phase 3 trial of rigosertib Oral in patients with transfusion-dependent lower risk MDS in the second half of 2014 at multiple sites in the United States and Europe.

We believe transfusion-dependent MDS is a serious unmet medical need, and the quality of life of these patients could be improved by lowering or eliminating transfusions. Approximately 77% of MDS patients are estimated to fall into the categories of MDS characterized as lower risk.

- *Combination therapy with rigosertib Oral and azacitidine in first-line MDS:* We are testing the combination of rigosertib Oral and azacitidine in a Phase 1/2 study of up to 40 first-line MDS patients. This trial will determine the maximum tolerated dose of rigosertib that can be given with azacitidine and assess the safety, tolerability, and activity of the combination. We expect to provide updates on this trial in the second half of 2014.
- *Rigosertib Oral in head and neck cancers:* We are evaluating rigosertib Oral in a Phase 2 trial in patients with head and neck cancers. Enrollment of patients in the first phase of the trial has been completed, and next steps in this patient population will be determined in the second quarter of 2014.
- *Rigosertib IV in combination with gemcitabine for first-line metastatic pancreatic cancer:* After conducting a pre-planned interim analysis of 165 patients in December 2013, we announced the discontinuation of this trial. The Data Safety Monitoring Board determined that the combination of rigosertib and gemcitabine is unlikely to demonstrate a statistically significant improvement in overall survival compared to gemcitabine alone.

We entered into a development and licensing agreement with Baxter in 2012 to commercialize rigosertib in Europe. In 2011, we entered into a licensing agreement with SymBio to commercialize rigosertib in Japan and Korea. We have retained development and commercialization rights to rigosertib in the rest of the world, including the United States. We will explore a variety of alternatives for the commercialization of rigosertib in territories we currently retain, including direct commercialization, co-promotion or selective out-licensing of rights to a third party.

Our second clinical-stage product candidate is ON 013105, or *briciclib*, a small molecule targeting an important regulatory protein, cyclin D1, which is often found at elevated levels in cancer cells. We are planning to resume Phase 1 clinical development of briciclib in 2014 with a multi-site dose escalation study in patients with advanced solid tumors refractory to current therapies.

Our third clinical-stage product candidate, *recilisib*, is being developed in collaboration with the U.S. Department of Defense, or DoD, for acute radiation syndromes, or ARS. We have conducted

animal efficacy studies and human Phase 1 clinical trials of recilisib. This product candidate is being developed under the FDA's Animal Efficacy Rule, which permits marketing approval for new medical countermeasures by relying on evidence from animal studies in appropriate animal models to support efficacy in humans. We have completed four Phase 1 trials to evaluate the safety and pharmacokinetics of recilisib in healthy human adult subjects using both subcutaneous and oral formulations, referred to as recilisib SC and recilisib Oral. We have received two orphan drug designations for recilisib for ARS in the United States.

In addition, we are also developing six preclinical stage molecules that target important enzymes or proteins involved in cell growth, metabolism or cell division.

We have broad-based capabilities that span drug discovery and clinical development, from medicinal chemistry and evaluation in biochemical, cell-based and animal models, through Phase 3 trials and regulatory filings in the United States, Europe and India. Our discovery program is based on a proprietary chemistry platform comprising more than 150 novel core chemical structures. Our chemistry and screening approaches aim to discover new drug candidates that kill cancer cells, with minimal toxicity to normal cells. Our intellectual property portfolio includes more than 100 issued patents and over 90 patent applications, either owned by us or licensed exclusively to us, including patents covering our most advanced product candidate, rigosertib. These patents and licenses cover composition-of-matter, manufacturing process, formulations and method-of-treatment claims for our clinical-stage product portfolio.

Our Strategy

We are committed to providing novel treatments to cancer patients, particularly in poorly served or unmet medical indications. We are focused on discovering and developing targeted small molecule anti-cancer product candidates. The key components of our strategy are to:

Seek Regulatory Approval for Rigosertib

- *For higher risk MDS patients who have failed azacitidine or decitabine therapy:* We reported top-line overall survival results from a Phase 3 trial with rigosertib IV in higher risk MDS patients in February 2014. We intend to discuss the results of this trial with regulatory authorities in the United States and Europe with the goal of determining next steps in advancing development of rigosertib IV in higher risk MDS.
- *For first-line treatment of transfusion-dependent lower risk MDS patients:* We reported response and safety data from the first Phase 2 trial of rigosertib Oral in transfusion-dependent lower risk patients at the ASH Annual Meeting in December 2013. Following regulatory input from the FDA, we expect to start our Phase 3 trial in this indication with rigosertib Oral in the second half of 2014.
- *For first-line treatment of MDS patients in combination with azacitidine:* We are testing the combination of rigosertib Oral and azacitidine (Vidaza®, a hypomethylating agent indicated for first-line treatment of MDS patients) in a Phase 1/2 study. We expect to provide updates on this trial in the second half of 2014.
- *For treatment of solid tumors:* We are currently evaluating rigosertib Oral in a Phase 2 trial as treatment for head and neck cancer. We expect to determine the next steps for this program in the second quarter of 2014.

Continue Development of Our Product Pipeline

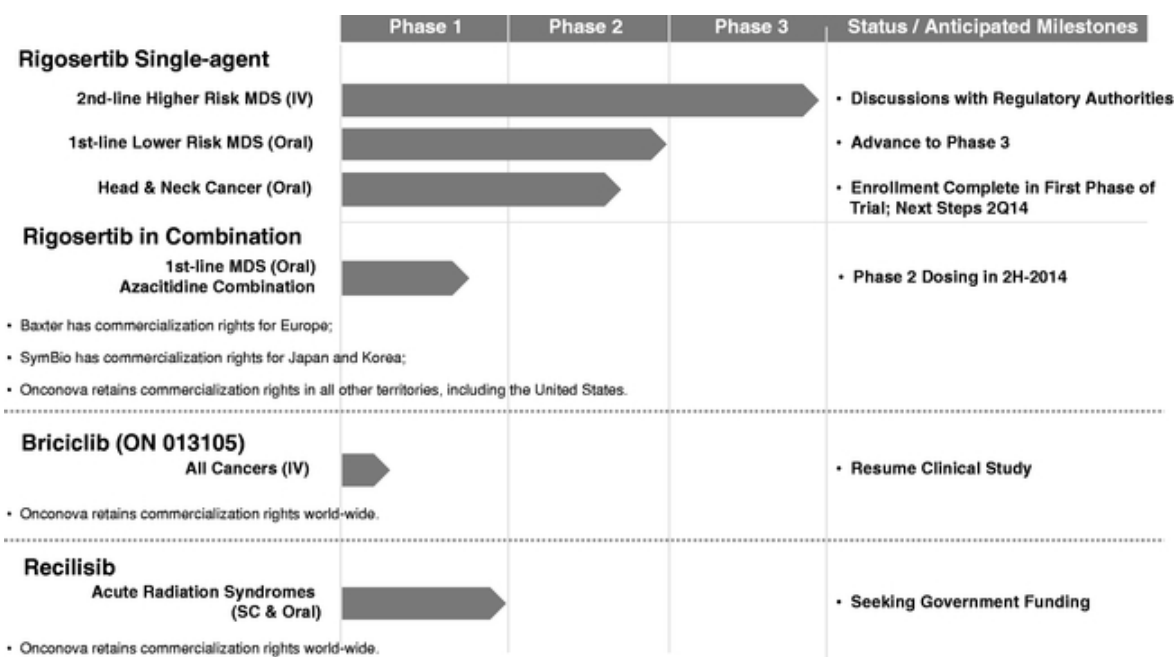
- **Initiate clinical development of briciclib for all tumor types:** We are planning to resume Phase 1 development of briciclib in 2014 with a multi-site dose escalation study in patients with advanced solid tumors refractory to current therapies.
- **Continue animal efficacy studies for recilisib:** We are seeking collaborations and government funding to support nonhuman primate efficacy studies as well as to identify predictive biomarkers in animals.
- **Advance a preclinical product candidate towards clinical testing:** Building on the experience and knowledge we have gained from our clinical-stage product candidates, we have identified several lead molecules in our preclinical pipeline. Two of these molecules are being developed in collaboration with GVK Biosciences, or GVK, a CRO based in India. We intend to explore additional collaborations to further the development of these product candidates.
- **Maintain Flexibility in Commercializing and Maximizing the Value of our Programs**
- While retaining U.S. and other territorial rights, we have entered into collaborations with Baxter to commercialize rigosertib in Europe and with SymBio to commercialize rigosertib in Japan and Korea. We will explore a variety of alternatives for the commercialization of rigosertib in territories we currently retain, including direct commercialization, co-promotion or selective out-licensing of rights to a third party.

Our Product Candidates

Clinical Programs

The development status of our three clinical-stage product candidates is summarized below:

Clinical-Stage Product Candidates



Rigosertib—Inhibitor of Phosphoinositide 3-Kinase & Polo-Like Kinase Pathways

Overview

Rigosertib is our most advanced product candidate and has been extensively tested in clinical trials, which have collectively enrolled more than 1,000 patients.

In February 2014, we reported that the Phase 3 ONTIME trial of rigosertib IV in patients with higher risk MDS who had progressed on, failed to respond to or relapsed after prior therapy with HMAs did not meet the primary endpoint of improving overall survival compared to BSC. However, a post-hoc analysis demonstrated a statistically significant increase in median overall survival in the subset of patients who had progressed on or failed to respond to previous treatment with HMAs, thus demonstrating potential activity of rigosertib in these MDS patients. Preliminary safety analysis from the trial indicates that rigosertib was generally well tolerated in the study population. We will consult with regulatory authorities to determine the next steps for rigosertib IV in higher risk MDS.

Rigosertib Oral, single agent, is being studied as first-line therapy in two Phase 2 trials in patients with transfusion-dependent lower risk MDS. We reported response and safety data from the first Phase 2 trial at the ASH Annual Meeting in December 2013. Following regulatory input from the FDA, we expect to start our Phase 3 trial in this indication in the second half of 2014. Rigosertib Oral is also being studied as a first-line therapy in combination with azacitidine in MDS in a Phase 1/2 study. We expect to provide updates on this trial in the second half of 2014.

Finally, we are evaluating rigosertib Oral in a Phase 2 trial in patients with head and neck cancers. Enrollment of patients in the first phase of the trial has been completed, and next steps in this indication will be determined in the second quarter of 2014.

Rigosertib Inhibits Signaling Pathways Associated with Cancer Cell Growth and Survival

Rigosertib inhibits two important cellular signaling pathways involving phosphoinositide 3-kinase, or PI3K, and polo-like kinase, or PLK, through a novel mechanism of action. PI3K signaling promotes the growth and survival of cells under stressful conditions, such as under low oxygen levels that are often found in tumors. By inhibiting the PI3K pathway in cancer cells, rigosertib promotes tumor cell apoptosis, or programmed cell death. The PLK pathway has a critical role in maintaining proper chromosome organization and sorting during cell division. By modulating the PLK pathway, rigosertib stops cancer cells at late stages of the cell division cycle, which leads to chromosome disorganization and death in these cells. In normal cells, rigosertib reversibly pauses progression of the cell cycle in the early stages, without causing harm or death to these cells. We recently presented new data on the mode of action of rigosertib. These data indicate that rigosertib binds to the Ras Binding Domain (RBD) found in many signaling proteins, including Raf kinases and PI3K. This specific binding disrupts protein-protein interactions necessary for signal transduction in the cell. This signal transduction inhibitory activity suggests novel ways in which cellular pathways could be interrupted by rigosertib and informs the design of future translational studies.

Myelodysplastic Syndromes

MDS is a group of blood disorders that affect bone marrow function. MDS typically affects patients over the age of 65. In MDS, bone marrow becomes dysplastic, or defective. The blood cells produced do not develop normally, such that too few healthy blood cells are released into the blood stream, which leads to low blood cell counts, or cytopenias. Thus, many patients with MDS require frequent blood transfusions. In most cases, the disease worsens and the patient develops progressive bone marrow failure. In advanced stages of the disease, immature blood cells, or blasts, leave the bone marrow and enter the blood stream, leading to acute myelogenous leukemia, or AML, which occurs in approximately one-third of patients with MDS.

Based on Surveillance Epidemiology and End Results (SEER) data from the National Cancer Institute, a marketing analytics firm has estimated the 2011 incidence of MDS at approximately 15,600 cases and the prevalence of MDS at approximately 52,000 cases in the United States. We believe that the actual incidence numbers may be higher, due to underdiagnosing and underreporting of new cases of MDS to centralized cancer registries, and that the incidence of MDS in the United States is likely to increase, due to an aging population, improved disease awareness and diagnostic precision, and an increase in the number of cases of secondary, often chemotherapy-induced, MDS.

MDS is typically diagnosed using routine blood tests or by observing symptoms, such as shortness of breath, weakness, easy bruising or bleeding, or fever with frequent infections. A diagnosis of MDS is confirmed by evaluating a bone marrow biopsy/aspirate showing dysplastic changes, and, in more advanced cases, the presence of excess blasts, meaning that blasts account for more than 5% of the total number of nucleated cells in the bone marrow. Because the bone marrow and blood cells in MDS patients can undergo different kinds of abnormal changes, several classification systems have been developed to gauge the severity of disease and help determine prognosis and treatment strategy. We use two standard classification systems, the French-American-British morphological classification system, or the FAB system, as modified by the World Health Organization, or WHO, and the International Prognostic Scoring System, or IPSS, to define patient inclusion criteria for our rigosertib trials in MDS:

- *FAB Classification/WHO Diagnostic Criteria.* In 1999, WHO modified the FAB system for MDS that had been based primarily on the percentage of blasts in the bone marrow and blood. Sub-categories under the WHO classification are: Refractory anemia, or RA (less than 5% blasts), RA with ringed sideroblasts, or RARS, refractory cytopenia with multilineage dysplasia, or RCMD, RA with excess blasts-1 (5-9% blasts), or RAEB-1, RAEB-2 (10-19% blasts), MDS with isolated deletion of the long arm of chromosome 5, or del(5q), and MDS unclassified, or MDS-U. Patients classified as RAEB in transformation (21-29% blasts), or RAEB-t, under the FAB system are reclassified in the WHO system as AML patients.
- *IPSS Diagnostic Criteria.* IPSS ranks the severity of chromosome abnormalities, number of cytopenias, and percentage of bone marrow blasts observed at diagnosis to calculate a four-level risk score: Low, Intermediate-1, Intermediate-2 and High. MDS patients are generally classified using IPSS in order to assess the risk of dying or having their disease progress to AML.

Patients with RAEB-1, RAEB-2 or RAEB-t under the FAB/WHO criteria or patients with IPSS risk scores of Intermediate-2 or High are generally considered to have higher risk MDS, with a median survival of less than two years. According to a marketing analytics firm, approximately 23% of MDS patients are classified in these higher risk categories.

Patients with IPSS scores of Low and Intermediate-1 are generally considered to have lower risk MDS, with an overall survival of approximately three to six years. Approximately 77% of MDS patients are classified in these lower risk categories.

Treating Myelodysplastic Syndromes

Stem cell or bone marrow transplantation is a potentially curative therapy for MDS. However, since most patients with MDS are elderly and therefore ineligible for transplantation, this option is generally considered only for the small proportion of younger MDS patients.

We believe that most higher risk and some lower risk MDS patients are treated with azacitidine and decitabine, the hypomethylating drugs approved in the United States for treatment of MDS. A provider of information, services and technology for the healthcare industry estimates that in the year ended June 2012, approximately 12,500 MDS patients in the United States received treatment with hypomethylating agents. For 2012, revenues of azacitidine were reported to be approximately

\$327 million in the United States and revenues for decitabine were reported to be approximately \$225 million in North America. Generic versions of azacitidine and decitabine entered the U.S. market in 2013.

A significant number of higher risk MDS patients fail or cannot tolerate treatment with azacitidine or decitabine, which represent the current standard of care for higher risk MDS patients, and almost all patients who initially respond to therapy eventually relapse. Median survival time of MDS patients who have failed hypomethylating drugs is less than six months. Accordingly, we believe that a new therapy that would extend survival in these patients would represent a major contribution in the treatment of MDS.

Hypomethylating drugs work by inhibiting the methylation of DNA. Methylation is a biochemical process involving the addition of a methyl group to DNA and plays an important role in gene expression during cell division and differentiation. By inhibiting methylation, hypomethylating drugs cause the death of rapidly dividing cells, including cancer cells that are no longer responsive to normal growth control mechanisms. Hypomethylation may also restore normal function to genes that are critical for differentiation and proliferation. The mechanisms underlying lack of response to and/or resistance to hypomethylating agents in patients are not well understood. Studies performed with decitabine in cultured cell lines revealed lowered expression of enzymes required for drug transport and activation.

By contrast, rigosertib works by inhibiting the PI3K pathway, a cellular signaling pathway that promotes the growth and survival of cells under stressful conditions, such as under low oxygen levels that are often found in tumors, and by modulating PLK pathway activity, which leads to chromosome disorganization and death in cancer cells. We believe that, because rigosertib has a mechanism of action that is different from hypomethylating agents, it may be active in patients who have failed treatment with those drugs.

In the case of lower risk MDS patients, those categorized as Low or Intermediate-1 risk with transfusion-dependent anemia and del(5q) cytogenetic abnormality are generally treated with lenalidomide (Revlimid®). For all other lower risk MDS patients, supportive care employing blood products, such as red blood cell and platelet transfusions, and erythroid stimulating agents, is the mainstay of therapy. Frequent transfusions introduce many risks, including iron overload, blood borne infections and immune-related reactions. We believe that a therapeutic agent that could lower or eliminate the need for transfusions over an extended period of time would fulfill a significant unmet medical need for this patient population.

Clinical Development of Rigosertib IV in Higher Risk Myelodysplastic Syndromes

We initiated clinical trials with rigosertib in MDS in 2008. We recently announced topline survival results from the multi-center Phase 3 ONTIME trial with rigosertib IV as a single agent in patients with MDS who had progressed on, failed to respond to or relapsed after prior therapy with HMAs. The ONTIME trial is a randomized, controlled study, where eligible patients must have progressed on, failed to respond to or relapsed after prior therapy with HMAs, have excess blasts (5-30% blasts) and have at least one cytopenia. There is currently no approved drug for this group of patients and the current standard treatment consists of best supportive care, which is treatment intended to manage disease-related symptoms. In the ONTIME trial, both groups of patients received best supportive care, with the treatment group of patients also receiving rigosertib. The study employed a 2:1 randomization in which two-thirds of the patients received rigosertib plus best supportive care, and one-third of patients received only best supportive care. The protocol for this trial was developed under an SPA. The EMA also provided Scientific Advice.

The primary objective of the ONTIME trial was to ascertain whether treatment with rigosertib leads to an overall survival benefit. Secondary objectives included evaluation of other responses, such as improvements in bone marrow and cytogenetic and blood profiles, according to standard response criteria for MDS defined by the 2006 WHO International Working Group, and to measure patients' quality of life scores and times to transition to AML. In this study, rigosertib was administered as a continuous intravenous infusion over a period of three days with a bag change every 24 hours. Patients used an ambulatory pump, avoiding the need for hospital stays. We completed enrollment in the ONTIME trial in July 2013.

The ONTIME trial was conducted at 42 sites in the United States and at 47 sites in Belgium, France, Germany, Italy and Spain. A total of 299 patients were enrolled in the trial. Among the 299 patients, 179 were enrolled at U.S. sites. In February 2014, we reported top-line overall survival results from this trial. Median overall survival in the rigosertib IV plus BSC arm was 8.2 months compared to 5.8 months in BSC only arm. Treatment with rigosertib IV plus BSC did not demonstrate a statistically significant improvement in median overall survival when compared to BSC only (Hazard Ratio=0.86; p-value=0.27). However, a post-hoc analysis demonstrated a statistically significant increase in median overall survival in the subset of patients who had progressed on or failed to respond to previous treatment with HMAs, thus demonstrating potential activity of rigosertib in these MDS patients. In this subset of patients (184 of 299 enrolled patients), the median overall survival was 8.5 months in the rigosertib IV plus BSC arm compared to 4.7 months in BSC only arm (Hazard Ratio=0.67; p-value=0.022). Severe adverse events were uncommon, with a similar profile of serious adverse events in both study arms. Grade 3/4 treatment-related hematologic and non-hematologic adverse events were reported in less than 7% and 3% of patients, respectively. Incidence of all grades of treatment-related nausea, diarrhea, fatigue and constipation were 22%, 17%, 17%, and 15%, respectively. All other treatment-related adverse events were reported in less than 10% of patients. Additional details, including secondary endpoints, will be presented at the 2014 American Society of Clinical Oncology (ASCO) Annual Meeting. During the second quarter of 2014, we plan to consult with regulatory authorities to determine the next steps for rigosertib IV in higher risk MDS.

Clinical Development of Rigosertib Oral in Lower Risk Myelodysplastic Syndromes

We filed an IND amendment with the FDA for rigosertib Oral in February 2009. Based on Phase 1 and Phase 2 trial results with rigosertib Oral, we believe that rigosertib has the potential to reduce or eliminate the transfusion needs and improve the quality of life for patients with lower risk MDS. As median survival for these transfusion-dependent patients is five or more years, frequent transfusions are subject to risks and limitations, including iron overload, blood borne infections and immune related reactions. We believe that an oral therapeutic agent that could lower or eliminate the need for transfusions over an extended period of time would fulfill a significant unmet medical need for this patient population.

We are enrolling transfusion-dependent lower risk MDS patients in two Phase 2 trials. We reported an updated interim analysis of response and safety data from the first Phase 2 trial, which we refer to as the "ONTARGET" trial, at the December 2013 ASH Annual Meeting. The ONTARGET trial is an open-label, multi-site trial testing the effect of rigosertib Oral in transfusion-dependent lower risk MDS patients. To be eligible for this study, patients must have received transfusions of at least four units of red blood cells during the eight weeks before randomization and patients can continue to receive transfusions and erythroid stimulating agents while in the trial. The primary objectives of this trial are to evaluate efficacy, as measured by transfusion independence, as well as safety.

Patients enrolled in the ONTARGET study received rigosertib Oral administered initially as 560 mg given two times daily (BID) for three weeks uninterrupted, later amended to 560 mg BID given intermittently (two weeks on and one week off), and then further reduced to 560 mg AM/280 mg PM given intermittently. The enrollment of 57 patients from five clinical sites was completed on

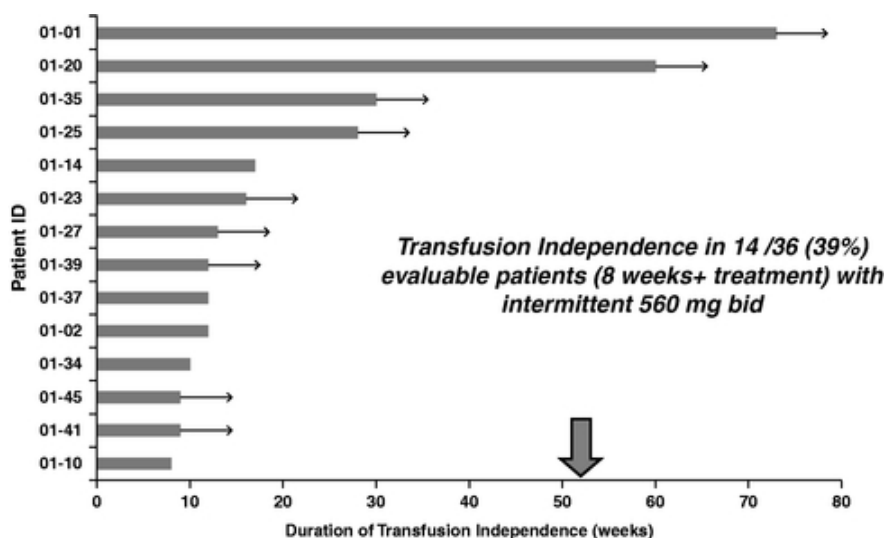
November 5, 2013. Updated interim results from 36 evaluable patients in the intermittent arm and nine patients from the uninterrupted arm were reported as a poster presentation during the ASH Annual Meeting in New Orleans in December 2013.

Evaluation of the data indicated that rigosertib Oral was generally well tolerated, with the most frequently observed drug-related side effects being urologic in nature and believed to be related to the dosing regimen. In the uninterrupted dosing arm of the study, six of the first nine patients experienced drug-related urinary side effects of Grade 2 or higher, meaning that they were more than mild in toxicity. Accordingly, the study protocol was amended to allow all patients to be treated with the intermittent dosing regimen. Urinary adverse events of grade 2+ were reported in 19 of 35 patients in the 560 mg BID intermittent dosing. Only one of 13 patients (8%) reported a Grade 2+ urinary event in the 560 mg AM/280 mg PM intermittent BID dosing.

Other adverse events of Grade 2 or higher reported in the study included intermittent neutropenia, or an abnormally low number of white blood cells that serve as the primary defense against infections, which occurred at Grade 3 in one patient and at Grade 4 in one other patient. Renal function was unaffected and gastrointestinal adverse events and fatigue were infrequently observed. Median duration of the treatment in the uninterrupted dosing arm was 23 weeks. Because the trial is ongoing, median duration of the treatment in the intermittent dosing (560 mg BID and 560 mg AM/280 mg PM) arm has not yet been determined. Initial response results showed that 14 of the 36 evaluable patients in the intermittent dosing arm and two of the eight evaluable patients in the uninterrupted dosing arm achieved transfusion independence, defined as a period of at least eight consecutive weeks without any red blood cell transfusions. As shown in the figure below, in the 14 patients who achieved transfusion independence in the intermittent dosing arm, the duration of transfusion independence ranged from eight weeks to more than 70 weeks.

Updated Interim Analysis of Transfusion-Dependent Lower Risk MDS Patients Treated with Rigosertib Oral in the Intermittent Dosing Arm

Transfusion Independence (14 of 36 Evaluable Patients)*



- Horizontal arrows indicate patients who continued to meet the criteria for transfusion independence at the time this data analysis was conducted. Transfusion independence is defined as a minimum of 8 consecutive weeks without red blood cell transfusions.

One of the aims of the ONTARGET trial was to correlate patient characteristics with transfusion response, thus potentially allowing for the selection of appropriate patients for future trials with rigosertib. To this end, a genomic scan for methylation was employed to analyze for correlations in response to rigosertib. A signature comprising 50 loci was identified, which related transfusion independence with methylation profile in 32 patients. A confirmation cohort of 20 additional patients is now being enrolled to further explore this potential prognostic tool.

We initiated a second multi-center Phase 2 trial in May 2013 in transfusion-dependent lower risk MDS patients who have failed treatment with ESAs, in which all patients will receive the intermittent dosing schedule. We expect to complete enrollment for this trial in the second half of 2014.

Previously, we conducted a Phase 1 trial of rigosertib Oral to determine drug safety and tolerability and to monitor plasma levels of rigosertib Oral in Low, Intermediate-1, Intermediate-2 and High-Risk MDS patients who had failed prior azacitidine, decitabine or lenalidomide therapy or injection of an ESA. Pharmacokinetic analysis showed that plasma levels of rigosertib could be achieved that were at or above levels predicted to be pharmacodynamically active. Encouraging preliminary signs of activity were observed. Safety data are available in 33 patients from this trial. The most frequent drug-related adverse events, occurring in at least 10% of patients, were decreased appetite, diarrhea, nausea and dysuria. Drug-related adverse events that were Grade 3 or greater in severity occurred in two patients and included urinary tract infection, syncope, or fainting, and dyspnea, or shortness of breath. Hematuria was identified as the most frequent dose-limiting toxicity, occurring in two patients.

Clinical Development of Rigosertib Oral in Head and Neck Cancers

According to the National Cancer Institute, head and neck cancers accounts for approximately 3% of all new cancer cases in the United States, with approximately 52,000 cases diagnosed in 2012. Single-modality treatment with surgery or radiation is generally recommended for the 30% to 40% of patients with early-stage disease. Combined modality therapy with surgery, radiation or concurrent chemotherapy and radiation is utilized for patients with locally or regionally advanced disease. Patients with very advanced or recurrent disease are treated with platinum-based chemotherapy, cetuximab (Erbix®) or both in combination. Expected overall survival in patients with head and neck cancers who have failed platinum-based therapy is about six months.

We filed an IND amendment with the FDA for rigosertib in head and neck cancer in November 2012. We conducted a Phase 1 trial with rigosertib Oral in 48 patients with various advanced solid tumors refractory to standard therapy, including six patients with head and neck cancers who had previously failed on platinum-based therapy. Two of these six head and neck cancers patients achieved durable responses to rigosertib therapy. One patient had a confirmed complete response, defined as the disappearance of chest and lung disease, and the other patient had a partial response, with a 53% decrease of liver metastasis. Both patients remained on therapy for an extended period of time, over 98 weeks for one and over 48 weeks for the other.

Rigosertib Oral is currently in a Phase 2 trial in relapsed or metastatic squamous cell carcinoma, with a focus on enrolling patients with head and neck cancers. The study will evaluate both human papilloma virus, or HPV, positive and HPV negative groups of patients. HPV infection presents a significant new therapeutic challenge in head and neck cancer patients. Enrollment of patients in the first phase of the trial has been completed, and next steps will be determined in this indication in the second quarter of 2014.

Briciclib—Targeted Anti-cancer Agent Modulating Cyclin D1

Overview

Briciclib suppresses cyclin D1 accumulation in cancer cells. Cyclin D1 is a protein required for normal progression through the cell reproduction cycle, and it is over-expressed in solid tumors and hematological diseases. We are planning to resume Phase 1 development of briciclib in 2014.

Clinical Development

We filed an IND with the FDA for briciclib in November 2008. We began a Phase 1 dose-escalation study of briciclib in patients with relapsed or refractory lymphomas and acute lymphoid leukemia, in which we were evaluating the safety, pharmacokinetics and activity of a once-weekly intravenous infusion regimen. In the first two complete dose cohorts, briciclib was readily detectible in plasma and had a half-life of less than one hour. In 2011, we suspended enrollment in our Phase 1 trial of briciclib because enrollment of patients was occurring too slowly and, as a result, inventory of briciclib clinical trial materials reached its expiration date. We are planning to resume Phase 1 clinical development of briciclib in 2014 with a multi-site dose escalation study in patients with advanced solid tumors refractory to current therapies.

Recilisib—Acute Radiation Syndromes Treatment

Overview

Recilisib is a small molecule with radiation protection properties. We are developing recilisib SC and recilisib Oral to address a need for medical countermeasures to treat the effects of ARS, specifically radiation-induced cytopenia. The DoD provided \$10.2 million in government funding to us pursuant to a number of programs through 2011. All agreements relating to the government funding of recilisib have expired and no funding or other obligations on the part of the DoD remain outstanding. Our strategy is to continue to seek support from government agencies and to develop recilisib under the FDA Animal Efficacy Rule.

Novel Mechanism of Action

Recilisib employs a novel mechanism of action that involves intracellular signaling and DNA damage repair pathways. In preclinical studies, cells treated with recilisib sustained less DNA damage upon exposure to ionizing radiation in comparison to untreated cells.

Clinical Development for Acute Radiation Syndromes

We filed original INDs with the FDA for recilisib SC and for recilisib Oral in April 2008 and May 2011, respectively. We have completed four Phase 1 trials with recilisib, three trials with recilisib SC in more than 50 healthy adults and one trial with recilisib Oral in nine healthy adults.

- In these Phase 1 trials, recilisib SC was generally well tolerated without significant drug-related systemic toxicity. Main adverse events were mild, self-limited injection site reactions, generally subsiding in a few hours. No clinically significant trends were noted in plasma cytokines between recilisib and placebo-treated groups. In one study, subjects successfully self-administered recilisib using an auto-injector system.
- Recilisib Oral displayed good bioavailability and was well tolerated. No drug-related systemic side-effects were observed. Drug concentration and overall exposure increased more than dose-proportionately, with corresponding decreases in the rate of clearance of recilisib.

Preclinical Development for Acute Radiation Syndromes

We have conducted preclinical studies to evaluate the radioprotective effects of recilisib SC and recilisib Oral in collaboration with the Armed Forces Radiobiology Research Institute and Georgetown University. In these studies, protection from radiation injury by recilisib was observed when administered prophylactically prior to ionizing radiation exposure in cellular and animal models. In irradiated mice, the protective benefits included increased overall survival and an enhanced rate of recovery of the hematopoietic system and crypt cells lining the gut. We are also working with the Biomedical Advanced Research and Development Authority on this program.

Preclinical Programs

In addition to our three clinical-stage product candidates, we have developed a pipeline of preclinical programs. Our preclinical pipeline includes six programs that target kinases, cellular metabolism or division. We intend to explore additional collaborations to further the development of these product candidates as we focus internally on our more advanced programs.

ON 1231320—Inhibitor of Polo-like Kinase 2, or PLK2: PLK2 is critical for centriole duplication during cell division, or mitosis. ON 1231320 is a specific inhibitor of PLK2, and in preclinical studies, it induced mitotic arrest and reduced tumor burden in mice injected subcutaneously with colon tumor and triple-negative breast cancer cells.

ON 123300—Inhibitor of the Cell Cycle and Cancer Cell Metabolism: ON 123300 inhibits the activity of two kinases, cyclin-dependent kinase 4, or CDK4, and AMP-activated protein kinase 5, or ARK5. CDK4 is an essential component of the cell division machinery and is a well-established target for therapeutic development. ARK5 is believed to be involved in the regulation of cancer cell metabolic activity. We believe that ON 123300 may have promise as a brain tumor therapy. We observed that ON 123300 can kill glioblastoma tumor cells in vitro and we also observed that, in mouse brain tumor models, ON 0123300 can cross the blood-brain barrier.

ON 108600—Inhibitor of Cyclin-dependent Kinase 9 and Casein Kinase 2: ON 108600 is a dual inhibitor of two growth-regulatory kinases. Cyclin-dependent kinase 9 is over expressed in several cancers, including leukemias and lymphomas. Casein kinase 2 is overexpressed in a variety of tumor types and plays a role in oncogenic processes including DNA damage and repair. We believe that ON 108600 may invoke a novel mechanism of cancer cell lethality by inhibiting these two targets.

ON 044580—Non-ATP Dual Inhibitor of Janus Kinase 2, or JAK-2, and Bcr-Abl Kinase: ON 044580 inhibits mutant forms of the two target kinases, including JAK2^{V617F} and imatinib-resistant Bcr-Abl^{T315I}. Three major myeloproliferative disorders, a group of diseases of the bone marrow in which excess cells are produced, are polycythemia vera, essential thrombocythemia and myeloid metaplasia with myelofibrosis. These disorders harbor mutations in JAK2, making this enzyme a potentially attractive therapeutic target for treating these disorders. Philadelphia chromosome-positive chronic myeloid leukemia cells make an abnormal protein called Bcr-Abl kinase, which is the clinical target of the approved inhibitor, imatinib (Gleevec®).

ON 24 Series of Compounds—Oral Anti-Tubulin Agents: Microtubules are organelles composed of a protein known as tubulin that help maintain cell shape, assist in cell movement and guide cell division. Interference with microtubule formation is an established anti-cancer strategy. ON 24 compounds cause tubulin to depolymerize, inducing mitotic arrest in cultured tumor cell lines.

ON 146 Series—Selective Inhibitors of PI3K alpha/delta Isoforms: Gain-of-function mutations in alpha and delta isoforms of PI3K are critical drivers of growth in several cancers. ON 146040 inhibits the growth of a variety of blood cancer cell lines, including Burkitt's lymphoma, MCL, multiple myeloma and chronic myeloid leukemia.

Our Proprietary Drug Discovery Platform

Our product candidates, designed for targeting cancer while protecting healthy cells, are derived from a novel chemistry platform and cell-based differential screening, which together define our discovery approach. Our chemical library contains more than 150 novel core chemical structures and thousands of unique compounds. Most are simple two- or three-ring structures that are built around a common core or signature element. Most kinase enzymes require the binding of adenosine triphosphate, or ATP, to function. Unlike most kinase inhibitors, our proprietary library includes many molecules that do not compete with the ATP binding site of kinases, which we believe may provide a more selective way to inhibit these enzymes.

Research and Development

Since commencing operations, we have dedicated a significant portion of our resources to the development of our clinical-stage product candidates, particularly rigosertib. We incurred research and development expenses of \$50.2 million, \$52.8 million, and \$22.6 million during the years ended December 31, 2013, 2012 and 2011, respectively. We anticipate that a significant portion of our operating expenses will continue to be related to research and development as we continue to advance our clinical-stage product candidates.

Collaborations

Baxter Healthcare SA

In September 2012, we entered into a development and license agreement with a subsidiary of Baxter International Inc., Baxter Healthcare SA, or Baxter, granting Baxter an exclusive, royalty-bearing license for the research, development, commercialization and manufacture (in specified instances) of rigosertib in all therapeutic indications in Europe. Under the Baxter agreement, we are obligated to use commercially reasonable efforts, in accordance with a development plan agreed upon by the parties, to direct, coordinate and manage the development of rigosertib for MDS and pancreatic cancer. In addition, there is a specified mechanism set forth in the agreement to expand the scope of the collaboration for additional indications. Our agreement with Baxter is guided by a joint steering committee. If the joint steering committee is not able to make a decision by consensus, then any dispute would be resolved by specified executive officers of both parties.

Under the terms of the agreement, Baxter made an upfront payment of \$50.0 million. We are eligible to receive pre-commercial milestone payments of up to an aggregate of \$337.5 million if specified development and regulatory milestones are achieved. The potential pre-commercial development milestone payments to us include the following:

- \$50.0 million for achievement of the primary endpoint of a Phase 3 clinical trial for rigosertib IV in higher risk MDS patients (the "MDS IV indication") or mutual agreement of the Parties to file for any marketing approval in either the European Union as a whole or in all of the Big Five EU Countries;
- \$25.0 million for the joint decision to proceed with the development of rigosertib for lower risk MDS; and
- \$25.0 million for each drug approval application filed for indications specified in the arrangement with Baxter.

We may also receive up to \$212.5 million in milestone payments for regulatory approvals of the rigosertib MDS indications specified in the arrangement with Baxter, each of which may be up to and in excess of \$100.0 million. We are also potentially eligible to receive an additional \$20.0 million pre-commercial milestone payment related to the timing of regulatory approval of rigosertib IV in

higher risk MDS patients in Europe. In addition to these pre-commercial milestones, we are eligible to receive up to an aggregate of \$250.0 million in milestone payments based on Baxter's achievement of pre-specified threshold levels of annual net sales of rigosertib. We are also entitled to receive royalties at percentage rates ranging from the low-teens to the low-twenties on net sales of rigosertib by Baxter in the licensed territory.

Under the agreement, Baxter is obligated to pay us royalties, on a country-by-country basis in the licensed territory, until the later of the expiration of all valid claims of the patent rights licensed to Baxter that cover the manufacture, use, sale or importation of rigosertib in such country, and the expiration of regulatory-based exclusivity for rigosertib in such country. If the patent rights and regulatory-based exclusivity expire in a particular country before a specified period of time after first commercial sale of rigosertib in that country, Baxter will pay us royalties at a reduced rate until the end of the specified period. In addition, unless we receive marketing approval for the use of rigosertib IV for MDS from the EMA or specified European Union countries without undertaking additional specified clinical-trials, the royalty rates may be reduced depending on when we receive marketing approval for the use of rigosertib IV for MDS from the EMA or specified European Union countries, and whether or not a competing product for refractory MDS has been approved within a specified period after our receipt of approval for rigosertib IV for MDS.

The agreement with Baxter will remain in effect until the expiration of all applicable royalty terms and satisfaction of all payment obligations in each licensed country, unless terminated earlier due to the uncured material breach or bankruptcy of a party, force majeure, or in the event of a specified commercial failure. We may terminate the agreement in the event that Baxter brings a challenge against us in relation to the licensed patents. Baxter may terminate the agreement without cause commencing after a specified period of time from the execution of the agreement.

In July 2012, Baxter purchased \$50.0 million of our Series J convertible preferred stock, which converted to shares of Common Stock immediately prior to the consummation of our IPO. Baxter also invested \$5.0 million in our initial public offering in July 2013.

SymBio Pharmaceuticals Limited

In July 2011, we entered into a license agreement with SymBio Pharmaceuticals Limited, or SymBio, as subsequently amended, granting SymBio an exclusive, royalty-bearing license for the development and commercialization of rigosertib in Japan and Korea. Under the SymBio license agreement, SymBio is obligated to use commercially reasonable efforts to develop and obtain market approval for rigosertib inside the licensed territory and we have similar obligations outside of the licensed territory. We have also entered into an agreement with SymBio to supply them with development-stage product. Under the SymBio license agreement we also agreed to supply commercial product to SymBio under specified terms that will be included in a commercial supply agreement to be negotiated prior to the first commercial sale of rigosertib. We have also granted SymBio a right of first negotiation to license or obtain the rights to develop and commercialize compounds having a chemical structure similar to rigosertib in the licensed territory.

Under the terms of the SymBio license agreement, we received an upfront payment of \$7.5 million. We are eligible to receive milestone payments of up to an aggregate of \$22.0 million from SymBio upon the achievement of specified development and regulatory milestones for specified indications. Of the regulatory milestones, \$5.0 million is due upon receipt of marketing approval in the United States of rigosertib IV in higher risk MDS patients, \$3.0 million is due upon receipt of marketing approval in Japan for rigosertib IV in higher risk MDS patients, \$5.0 million is due upon receipt of marketing approval in the United States for rigosertib Oral in lower risk MDS patients, \$5.0 million is due upon receipt of marketing approval in Japan for rigosertib Oral in lower risk MDS patients. Furthermore, upon receipt of marketing approval in the United States and Japan for an

additional specified indication of rigosertib, which we are currently not pursuing, an aggregate of \$4.0 million would be due. In addition to these pre-commercial milestones, we are eligible to receive tiered milestone payments of up to an aggregate of \$30.0 million based upon annual net sales of rigosertib by SymBio in the licensed territory. Further, under the terms of the SymBio license agreement, SymBio is obligated to make royalty payments to us at percentage rates ranging from the mid-teens to 20% based on net sales of rigosertib by SymBio in the licensed territory.

Royalties will be payable under the SymBio agreement on a country-by-country basis in the licensed territory, until the later of the expiration of marketing exclusivity in those countries, a specified period of time after first commercial sale of rigosertib in such country, or the expiration of all valid claims of the licensed patents covering rigosertib or the manufacture or use of rigosertib in such country. If no valid claim exists covering the composition of matter of rigosertib or the use of or treatment with rigosertib in a particular country before the expiration of the royalty term, and specified competing products achieve a specified market share percentage in such country, SymBio's obligation to pay us royalties will continue at a reduced royalty rate until the end of the royalty term. In addition, the applicable royalties payable to us may be reduced if SymBio is required to pay royalties to third parties for licenses to intellectual property rights necessary to develop, use, manufacture or commercialize rigosertib in the licensed territory.

The license agreement with SymBio will remain in effect until the expiration of the royalty term. However, the SymBio license agreement may be terminated earlier due to the uncured material breach or bankruptcy of a party, or force majeure. If SymBio terminates the license agreement in these circumstances, its licenses to rigosertib will survive, subject to SymBio's milestone and royalty obligations, which SymBio may elect to defer and offset against any damages that may be determined to be due from us. In addition, we may terminate the license agreement in the event that SymBio brings a challenge against us in relation to the licensed patents, and SymBio may terminate the license agreement without cause by providing us with written notice a specified period of time in advance of termination.

The Leukemia and Lymphoma Society

In May 2010, we entered into a funding agreement with The Leukemia and Lymphoma Society, or LLS, to fund the development of rigosertib. Under the LLS funding agreement, we are obligated to use the funding received exclusively for the payment or reimbursement of the costs and expenses for clinical development activities for rigosertib. Under this agreement, we retain ownership and control of all intellectual property pertaining to works of authorship, inventions, know-how, information, data and proprietary material.

Under the LLS funding agreement, as amended, we received funding of \$8.0 million from LLS through 2012. We did not receive any funding from LLS in 2013 and we terminated the funding agreement effective as of March 2013. We are required to make specified payments to LLS, including payments payable upon execution of the first out-license; first approval for marketing by a regulatory body; completion of the first commercial sale of rigosertib; and achieving specified annual net sales levels of rigosertib. The extent of these payments and our obligations will depend on whether we out-license rights to develop or commercialize rigosertib to a third party, we commercialize rigosertib on our own or we combine with or are sold to another company. In addition, we will pay to LLS a single-digit percentage royalty of our net sales of rigosertib, if any. The sum of our payments to LLS is capped at three times the total funding received from LLS, or \$24.0 million.

In addition, some of our obligations under the funding agreement will remain in effect until the completion of specified milestones and payments to LLS. Assuming the successful outcome of the development activities covered by the LLS funding agreement and our receipt of necessary regulatory approvals, we will be required to take commercially reasonable steps through March 2018 to advance

the development of rigosertib in clinical trials and to bring rigosertib to practical application for MDS in a major market country, provided that we reasonably believe the product is safe and effective. We believe that we can satisfy our obligation by out-licensing rigosertib to, or partnering rigosertib with, a third party. We are required to report to LLS on our efforts and results with respect to continuing development of rigosertib. Our failure to perform these diligence obligations, even if we successfully achieve the specified development milestones, would require us to pay back to LLS the total amount of the funding we received from them, unless an exception applies. If LLS were to claim that such failure occurred and we disagreed with such claim, the dispute would be settled through binding arbitration.

Preclinical Collaboration

In December 2012, we formed GBO, LLC, or GBO, an entity jointly-owned by both us and GVK Biosciences Private Limited, or GVK, to collaborate on the development of two of our preclinical programs. GVK made an initial capital contribution of \$500,000 in exchange for a 10% interest in GBO and we contributed a sub-license to the intellectual property related to the two programs in exchange for a 90% interest. GVK will be required to make additional capital contributions over time, subject to specified conditions, and its interest in GBO will increase to as much as 50%. At specified times, we will be entitled to buy back from GVK the rights to either of these two programs.

Intellectual Property

Patents and Proprietary Rights

Our intellectual property is derived through our internal research, licensing agreements with Temple University, or Temple, and licensing research agreements with the Mount Sinai School of Medicine, or Mount Sinai.

License Agreement with Temple University

In January 1999, we entered into a license agreement with Temple as subsequently amended, to obtain an exclusive, world-wide license to certain Temple patents and technical information to make, have made, use, sell, offer for sale and import several classes of novel compounds, including our three clinical-stage product candidates, rigosertib, briciclib and recilisib.

Under the terms of the license agreement, we paid Temple a non-refundable up-front payment, and are required to pay annual license maintenance fees, as well as a low single-digit percentage of net sales as a royalty. In addition, we agreed to pay Temple 25% of any consideration received from any sublicensee of the licensed Temple patents and technical information, which does not include any royalties on sales, funds received for research and development or proceeds from any equity or debt investment.

The license agreement with Temple can be terminated by mutual agreement or due to the material breach or bankruptcy of either party. We may terminate the license agreement for any reason by giving Temple prior written notice.

Research Agreement with Mount Sinai School of Medicine

In May 2010, we entered into a research agreement with Mount Sinai. This agreement is described in more detail under the caption "Certain Relationships and Related Party Transactions—Research Agreement."

Rigosertib Patents

As of May 6, 2013, we owned or exclusively licensed 64 issued patents and 18 pending patent applications covering composition-of-matter, process, formulation and various indications for

method-of-use for rigosertib filed worldwide, including five patents and three patent applications in the United States. The U.S. composition-of-matter patent for rigosertib, which we in-licensed pursuant to the license agreement with Temple, expires in 2026. The U.S. method of treatment patent for rigosertib, which we also in-licensed from Temple, expires in 2025.

Briciclib Patents

As of May 6, 2013, we owned or exclusively licensed eight issued patents and five pending patent applications covering composition-of-matter, process, formulation and various indications for method-of-use for briciclib filed worldwide, including one patent in the United States. The U.S. composition-of-matter patent for briciclib expires in 2025.

Recilisib Patents

As of May 6, 2013, we owned or exclusively licensed 43 issued patents and 38 pending patent applications covering composition of matter, formulation and various indications for method-of-use for recilisib filed worldwide, including four patents and five patent applications in the United States. The U.S. composition-of-matter patent for recilisib expires in 2020.

General Considerations

As with other biotechnology and pharmaceutical companies, our ability to maintain and solidify a proprietary position for our product candidates will depend upon our success in obtaining effective patent claims and enforcing those claims once granted.

Our commercial success will depend in part upon not infringing upon the proprietary rights of third parties. It is uncertain whether the issuance of any third-party patent would require us to alter our development or commercial strategies, or our product candidates or processes, obtain licenses or cease certain activities. The biotechnology and pharmaceutical industries are characterized by extensive litigation regarding patents and other intellectual property rights. If a third party commences a patent infringement action against us, or our collaborators, it could consume significant financial and management resources, regardless of the merit of the claims or the outcome of the litigation.

The term of a patent that covers an FDA-approved drug may be eligible for additional patent term extension, which provides patent term restoration as compensation for the patent term lost during the FDA regulatory review process. The Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act, permits a patent term extension of up to five years beyond the expiration of the patent. The length of the patent term extension is related to the length of time the drug is under regulatory review. Patent extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval and only one patent applicable to an approved drug may be extended. Similar provisions are available in Europe and other foreign jurisdictions to extend the term of a patent that covers an approved drug. In the future, if and when our pharmaceutical products receive FDA approval, we expect to apply for patent term extensions on patents covering those products.

Furthermore, we may be able to obtain extension of patent term by adjustment of the said term under the provisions of 35 U.S.C. § 154 if the issue of an original patent is delayed due to the failure of the U.S. Patent and Trademark Office. For example, we have received adjustments of 1,139 days extension to the patent term for the rigosertib composition of matter patent (US 7,598,232), 1,155 days extension for the patent covering the process for making rigosertib (US 8,143,453) and 751 days extension for rigosertib formulation patent (US 8,063,109) under the provisions of 35 U.S.C. §154.

Many pharmaceutical companies, biotechnology companies and academic institutions are competing with us in the field of oncology and filing patent applications potentially relevant to our business. Even when a third party patent is identified, we may conclude upon a thorough analysis, that we do not infringe upon the patent or that the patent is invalid. If the third-party patent owner disagrees with our conclusion and we continue with the business activity in question, we may be subject to patent litigation. Alternatively, we might decide to initiate litigation in an attempt to have a court declare the third-party patent invalid or non-infringed by our activity. In either scenario, patent litigation typically is costly and time-consuming, and the outcome can be favorable or unfavorable.

In addition to patents, we rely upon unpatented trade secrets, know-how and continuing technological innovation to develop and maintain a competitive position. We seek to protect our proprietary information, in part, through confidentiality agreements with our employees, collaborators, contractors and consultants, and invention assignment agreements with our employees. We also have agreements requiring assignment of inventions with selected consultants and collaborators. The confidentiality agreements are designed to protect our proprietary information and, in the case of agreements or clauses requiring invention assignment, to grant us ownership of technologies that are developed through a relationship with a third party.

Competition

The pharmaceutical industry is highly competitive and subject to rapid and significant technological change. While we believe that our development experience and scientific knowledge provide us with competitive advantages, we face competition from both large and small pharmaceutical and biotechnology companies. There are a number of pharmaceutical companies, biotechnology companies, public and private universities and research organizations actively engaged in the research and development of products that may compete with our products. Many of these companies are multinational pharmaceutical or biotechnology organizations, which are pursuing the development of, or are currently marketing, pharmaceuticals that target the key oncology indications or cellular pathways on which we are focused.

It is probable that the increasing incidence and prevalence of cancer will lead to many more companies seeking to develop products and therapies for the treatment of unmet needs in oncology. Many of our competitors have significantly greater financial, technical and human resources than we have. Many of our competitors also have a significant advantage with respect to experience in the discovery and development of product candidates, as well as obtaining FDA and other regulatory approvals of products and the commercialization of those products. We anticipate intense and increasing competition as new drugs enter the market and as more advanced technologies become available. Our success will be based in part on our ability to identify, develop and manage a portfolio of drugs that are safer and more effective than competing products in the treatment of cancer patients.

Myelodysplastic Syndromes

There are several ongoing clinical trials aimed at expanding the use of approved chemotherapeutic and immunomodulatory agents in higher risk MDS, as well as several new clinical programs testing novel technologies in this area. Companies competing in this space include Eisai Inc. (decitabine), Celgene Corporation (azacitidine in combination with lenalidomide or vorinostat (Zolinza®), Cell Therapeutics, Inc. (tosedostat in combination with decitabine or cytarabine), Cyclacel Pharmaceuticals, Inc. (sapacitabine), Mirati Therapeutics (mocetinostat in combination with azacitidine), and MEI Pharma (pacrinostat in combination with azacitidine). To our knowledge, there are no Phase 3 trials other than our trial for rigosertib IV being conducted for higher risk MDS patients who have failed treatment with hypomethylating agents. In the lower risk MDS market, we face competition from a number of companies in mid-stage clinical trials, such as Celgene Corporation (lenalidomide), Array BioPharma Inc (ARRY-614), and Acceleron Pharma (sotatercept and ACE-536).

Acute Radiation Syndrome

Competitors developing products to address ARS include Soligenix, Inc., Cellerant Therapeutics, Inc., and Cleveland BioLabs, Inc. Each of these companies is working with the U.S. government to develop its products through federal contracts and grants.

Manufacturing

Our product candidates are synthetic small molecules. Manufacturing activities must comply with FDA current good manufacturing practices, or cGMP, regulations. We conduct our manufacturing activities under individual purchase orders with third-party contract manufacturers, or CMOs. We have in place quality agreements with our key CMOs and are negotiating supply agreements with them. We have also established an internal quality management organization, which audits and qualifies CMOs in the United States and abroad.

We are working with CMOs to produce the rigosertib active pharmaceutical ingredient, which we believe will enable us to launch and commercialize rigosertib IV if and when marketing approval is obtained. Other CMOs produce rigosertib IV and rigosertib Oral for use in our clinical trials. We believe that the manufacturing processes for the active pharmaceutical ingredient and finished drug products for rigosertib have been developed to adequately support future development and commercial demands.

The FDA regulates and inspects equipment, facilities and processes used in manufacturing pharmaceutical products prior to approval. If we fail to comply with applicable cGMP requirements and conditions of product approval, the FDA may seek sanctions, including fines, civil penalties, injunctions, suspension of manufacturing operations, operating restrictions, withdrawal of FDA approval, seizure or recall of products and criminal prosecution. Although we periodically monitor the FDA compliance of our third-party CMOs, we cannot be certain that our present or future third-party CMOs will consistently comply with cGMP and other applicable FDA regulatory requirements.

Commercial Operations

We do not currently have an organization for the sales, marketing and distribution of pharmaceutical products. We may rely on licensing and co-promotion agreements with strategic partners for the commercialization of our products in the United States and other territories. If we choose to build a commercial infrastructure to support marketing in the United States, such commercial infrastructure could be expected to include a targeted, oncology sales force supported by sales management, internal sales support, an internal marketing group and distribution support. To develop the appropriate commercial infrastructure internally, we would have to invest financial and management resources, some of which would have to be deployed prior to any confirmation that rigosertib will be approved.

Government Regulation

As a pharmaceutical company that operates in the United States, we are subject to extensive regulation by the FDA, and other federal, state, and local regulatory agencies. The Federal Food, Drug, and Cosmetic Act, or the FDC Act, and its implementing regulations set forth, among other things, requirements for the research, testing, development, manufacture, quality control, safety, effectiveness, approval, labeling, storage, record keeping, reporting, distribution, import, export, advertising and promotion of our products. Although the discussion below focuses on regulation in the United States, we anticipate seeking approval for, and marketing of, our products in other countries. Generally, our activities in other countries will be subject to regulation that is similar in nature and scope as that imposed in the United States, although there can be important differences. Additionally, some significant aspects of regulation in Europe are addressed in a centralized way through the EMA, but

country-specific regulation remains essential in many respects. The process of obtaining regulatory marketing approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources.

United States Government Regulation

The FDA is the main regulatory body that controls pharmaceuticals in the United States, and its regulatory authority is based in the FDC Act. Pharmaceutical products are also subject to other federal, state and local statutes. A failure to comply explicitly with any requirements during the product development, approval, or post-approval periods, may lead to administrative or judicial sanctions. These sanctions could include the imposition by the FDA or an institutional review board, or IRB, of a hold on clinical trials, refusal to approve pending marketing applications or supplements, withdrawal of approval, warning letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, civil penalties or criminal prosecution.

The steps required before a new drug may be marketed in the United States generally include:

- Completion of preclinical laboratory tests, animal studies and formulation studies in compliance with the FDA's GLP regulations;
- Submission to the FDA of an IND to support human clinical testing;
- Approval by an IRB at each clinical site before each trial may be initiated;
- Performance of adequate and well-controlled clinical trials in accordance with federal regulations and with current good clinical practices, or GCPs, to establish the safety and efficacy of the investigational drug product for each targeted indication;
- Submission of an NDA to the FDA;
- Satisfactory completion of an FDA Advisory Committee review, if applicable;
- Satisfactory completion of an FDA inspection of the manufacturing facilities at which the investigational product is produced to assess compliance with cGMP, and to assure that the facilities, methods and controls are adequate; and
- FDA review and approval of the NDA.

Clinical Trials

An IND is a request for authorization from the FDA to administer an investigational drug product to humans. This authorization is required before interstate shipping and administration of any new drug product to humans that is not the subject of an approved NDA. A 30-day waiting period after the submission of each IND is required prior to the commencement of clinical testing in humans. If the FDA has neither commented on nor questioned the IND within this 30-day period, the clinical trial proposed in the IND may begin. Clinical trials involve the administration of the investigational drug to patients under the supervision of qualified investigators following GCPs, an international standard meant to protect the rights and health of patients and to define the roles of clinical trial sponsors, administrators and monitors. Clinical trials are conducted under protocols that detail the parameters to be used in monitoring safety, and the efficacy criteria to be evaluated. Each protocol involving testing on U.S. patients and subsequent protocol amendments must be submitted to the FDA as part of the IND. The informed written consent of each participating subject is required. The clinical investigation of an investigational drug is generally divided into three phases. Although the phases are usually

conducted sequentially, they may overlap or be combined. The three phases of an investigation are as follows:

- Phase 1. Phase 1 includes the initial introduction of an investigational drug into humans. Phase 1 clinical trials may be conducted in patients with the target disease or condition or healthy volunteers. These studies are designed to evaluate the safety, metabolism, pharmacokinetics and pharmacologic actions of the investigational drug in humans, the side effects associated with increasing doses, and if possible, to gain early evidence on effectiveness. During Phase 1 clinical trials, sufficient information about the investigational product's pharmacokinetics and pharmacological effects may be obtained to permit the design of Phase 2 clinical trials. The total number of participants included in Phase 1 clinical trials varies, but is generally in the range of 20 to 80.
- Phase 2. Phase 2 includes the controlled clinical trials conducted to evaluate the effectiveness of the investigational product for a particular indication(s) in patients with the disease or condition under study, to determine dosage tolerance and optimal dosage, and to identify possible adverse side effects and safety risks associated with the drug. Phase 2 clinical trials are typically well-controlled, closely monitored, and conducted in a limited patient population, usually involving no more than several hundred participants.
- Phase 3. Phase 3 clinical trials are controlled clinical trials conducted in an expanded patient population at geographically dispersed clinical trial sites. They are performed after preliminary evidence suggesting effectiveness of the investigational product has been obtained, and are intended to further evaluate dosage, clinical effectiveness and safety, to establish the overall benefit-risk relationship of the product, and to provide an adequate basis for product approval. Phase 3 clinical trials usually involve several hundred to several thousand participants. In most cases, the FDA requires two adequate and well controlled Phase 3 clinical trials to demonstrate the efficacy of the drug. A single Phase 3 trial with other confirmatory evidence may be sufficient in rare instances where the study is a large multicenter trial demonstrating internal consistency and a statistically very persuasive finding of a clinically meaningful effect on mortality, irreversible morbidity or prevention of a disease with a potentially serious outcome and confirmation of the result in a second trial would be practically or ethically impossible.

The decision to terminate development of an investigational drug product may be made by either a health authority body, such as the FDA or IRB/ethics committees, or by a company for various reasons. The FDA may order the temporary, or permanent, discontinuation of a clinical trial at any time, or impose other sanctions, if it believes that the clinical trial either is not being conducted in accordance with FDA requirements or presents an unacceptable risk to the clinical trial patients. In some cases, clinical trials are overseen by an independent group of qualified experts organized by the trial sponsor, or the clinical monitoring board. This group provides authorization for whether or not a trial may move forward at designated check points. These decisions are based on the limited access to data from the ongoing trial. The suspension or termination of development can occur during any phase of clinical trials if it is determined that the participants or patients are being exposed to an unacceptable health risk. In addition, there are requirements for the registration of ongoing clinical trials of drugs on public registries and the disclosure of certain information pertaining to the trials as well as clinical trial results after completion.

A sponsor may be able to request an SPA, the purpose of which is to reach agreement with the FDA on the Phase 3 clinical trial protocol design and analysis that will form the primary basis of an efficacy claim. A sponsor meeting the regulatory criteria may make a specific request for an SPA and provide information regarding the design and size of the proposed clinical trial. An SPA request must be made before the proposed trial begins, and all open issues must be resolved before the trial begins. If a written agreement is reached, it will be documented and made part of the record. The agreement

will be binding on the FDA and may not be changed by the sponsor or the FDA after the trial begins except with the written agreement of the sponsor and the FDA or if the FDA determines that a substantial scientific issue essential to determining the safety or efficacy of the product candidate was identified after the testing began. An SPA is not binding if new circumstances arise, and there is no guarantee that a study will ultimately be adequate to support an approval even if the study is subject to an SPA. Rigosertib is being tested in several advanced stage clinical trials, including a pivotal Phase 3 trial under an SPA. Having an SPA agreement does not guarantee that a product will receive FDA approval.

Assuming successful completion of all required testing in accordance with all applicable regulatory requirements, detailed investigational drug product information is submitted to the FDA in the form of a NDA to request market approval for the product in specified indications.

New Drug Applications

In order to obtain approval to market a drug in the United States, a marketing application must be submitted to the FDA that provides data establishing the safety and effectiveness of the drug product for the proposed indication. The application includes all relevant data available from pertinent preclinical and clinical trials, including negative or ambiguous results as well as positive findings, together with detailed information relating to the product's chemistry, manufacturing, controls and proposed labeling, among other things. Data can come from company-sponsored clinical trials intended to test the safety and effectiveness of a product, or from a number of alternative sources, including studies initiated by investigators. To support marketing approval, the data submitted must be sufficient in quality and quantity to establish the safety and effectiveness of the investigational drug product to the satisfaction of the FDA.

In most cases, the NDA must be accompanied by a substantial user fee (currently exceeding \$1,958,000); there may be some instances in which the user fee is waived. The FDA will initially review the NDA for completeness before it accepts the NDA for filing. The FDA has 60 days from its receipt of an NDA to determine whether the application will be accepted for filing based on the agency's threshold determination that it is sufficiently complete to permit substantive review. After the NDA submission is accepted for filing, the FDA begins an in-depth review. The FDA has agreed to certain performance goals in the review of NDAs. Most such applications for standard review drug products are reviewed within ten to twelve months. The FDA can extend this review by three months to consider certain late-submitted information or information intended to clarify information already provided in the submission. The FDA reviews the NDA to determine, among other things, whether the proposed product is safe and effective for its intended use, and whether the product is being manufactured in accordance with cGMP. The FDA may refer applications for novel drug products which present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

Before approving an NDA, the FDA will inspect the facilities at which the product is manufactured. The FDA will not approve the product unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA, the FDA will typically inspect one or more clinical sites to assure compliance with GCP. After the FDA evaluates the NDA and the manufacturing facilities, it issues either an approval letter or a complete response letter. A complete response letter generally outlines the deficiencies in the submission and may require substantial additional testing or information in order for the FDA to reconsider the application. If, or when, those deficiencies have been addressed to the FDA's satisfaction in a resubmission of the NDA, the FDA will issue an approval letter. The FDA has committed to reviewing

such resubmissions in two or six months depending on the type of information included. Notwithstanding the submission of any requested additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval.

An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications. As a condition of NDA approval, the FDA may require a risk evaluation and mitigation strategy, or REMS, to help ensure that the benefits of the drug outweigh the potential risks. REMS can include medication guides, communication plans for healthcare professionals, and elements to assure safe use, or ETASU. ETASU can include, but are not limited to, special training or certification for prescribing or dispensing, dispensing only under certain circumstances, special monitoring, and the use of patient registries. The requirement for a REMS can materially affect the potential market and profitability of the drug. Moreover, product approval may require substantial post-approval testing and surveillance to monitor the drug's safety or efficacy. Once granted, product approvals may be withdrawn if compliance with regulatory standards is not maintained or problems are identified following initial marketing.

Changes to some of the conditions established in an approved application, including changes in indications, labeling, or manufacturing processes or facilities, require submission and FDA approval of a new NDA or NDA supplement before the change can be implemented. An NDA supplement for a new indication typically requires clinical data similar to that in the original application, and the FDA uses the same procedures and actions in reviewing NDA supplements as it does in reviewing NDAs.

Advertising and Promotion

The FDA and other federal regulatory agencies closely regulate the marketing and promotion of drugs through, among other things, standards and regulations for direct-to-consumer advertising, communications regarding unapproved uses, industry-sponsored scientific and educational activities, and promotional activities involving the Internet. A product cannot be commercially promoted before it is approved. After approval, product promotion can include only those claims relating to safety and effectiveness that are consistent with the labeling approved by the FDA. Healthcare providers are permitted to prescribe drugs for "off-label" uses—that is, uses not approved by the FDA and therefore not described in the drug's labeling—because the FDA does not regulate the practice of medicine. However, FDA regulations impose stringent restrictions on manufacturers' communications regarding off-label uses. Broadly speaking, a manufacturer may not promote a drug for off-label use, but may engage in non-promotional, balanced communication regarding off-label use under specified conditions. Failure to comply with applicable FDA requirements and restrictions in this area may subject a company to adverse publicity and enforcement action by the FDA, the DOJ, or the Office of the Inspector General of HHS, as well as state authorities. This could subject a company to a range of penalties that could have a significant commercial impact, including civil and criminal fines and agreements that materially restrict the manner in which a company promotes or distributes drug products.

Post-Approval Regulations

After regulatory approval of a drug is obtained, a company is required to comply with a number of post-approval requirements. For example, as a condition of approval of an NDA, the FDA may require post-marketing testing, including Phase 4 clinical trials, and surveillance to further assess and monitor the product's safety and effectiveness after commercialization. Regulatory approval of oncology products often requires that patients in clinical trials be followed for long periods to determine the overall survival benefit of the drug. In addition, as a holder of an approved NDA, a company would be required to report adverse reactions and production problems to the FDA, to provide updated safety and efficacy information, and to comply with requirements concerning advertising and promotional labeling for any of its products. Also, quality control and manufacturing procedures must continue to

conform to cGMP after approval to assure and preserve the long term stability of the drug or biological product. The FDA periodically inspects manufacturing facilities to assess compliance with cGMP, which imposes extensive procedural and substantive record keeping requirements. In addition, changes to the manufacturing process are strictly regulated, and, depending on the significance of the change, may require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP and impose reporting and documentation requirements upon a company and any third-party manufacturers that a company may decide to use. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain compliance with cGMP and other aspects of regulatory compliance.

We rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of our product candidates. Future FDA and state inspections may identify compliance issues at our facilities or at the facilities of our contract manufacturers that may disrupt production or distribution, or require substantial resources to correct. In addition, discovery of previously unknown problems with a product or the failure to comply with applicable requirements may result in restrictions on a product, manufacturer or holder of an approved NDA, including withdrawal or recall of the product from the market or other voluntary, FDA-initiated or judicial action that could delay or prohibit further marketing.

Newly discovered or developed safety or effectiveness data may require changes to a product's approved labeling, including the addition of new warnings and contraindications, and also may require the implementation of other risk management measures. Also, new government requirements, including those resulting from new legislation, may be established, or the FDA's policies may change, which could delay or prevent regulatory approval of our products under development.

FDA Animal Efficacy Rule for Approval of Medical Countermeasures

Marketing approval by the FDA for new medical countermeasures in situations for which human efficacy testing is not feasible or ethical, such as for ARS, is based on the so-called "Animal Efficacy Rule." Under this rule, FDA can rely on the evidence from animal studies to provide substantial prediction of effectiveness of an agent in humans, when coupled with:

- a reasonably well understood pathophysiological mechanism for the toxicity of the radiological or nuclear substance and its amelioration or prevention by the agent;
- protective effect is demonstrated in generally more than one animal species expected to react with a response predictive for humans, and hence be a reliable indicator of its effectiveness in humans;
- animal study endpoint is clearly related to the desired benefit in humans; and
- data or information on the pharmacokinetics and pharmacodynamics of the product in animals and humans is sufficiently well understood to allow selection of a dose predicted to be effective in humans.

The Hatch-Waxman Amendments to the FDC Act

Orange Book Listing

In seeking approval for a drug through an NDA, applicants are required to list with the FDA each patent whose claims cover the applicant's product. Upon approval of a drug, each of the patents listed in the application for the drug is then published in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations, commonly known as the Orange Book. Drugs listed in the Orange Book can, in turn, be cited by potential generic competitors in support of approval of an abbreviated new drug application, or ANDA. An ANDA provides for marketing of a drug product that

has the same active ingredients in the same strengths and dosage form as the listed drug and has been shown through bioequivalence testing to be therapeutically equivalent to the listed drug. Other than the requirement for bioequivalence testing, ANDA applicants are not required to conduct, or submit results of, pre-clinical or clinical tests to prove the safety or effectiveness of their drug product. Drugs approved in this way are commonly referred to as "generic equivalents" to the listed drug, and can often be substituted by pharmacists under prescriptions written for the original listed drug. The ANDA applicant is required to certify to the FDA concerning any patents listed for the approved product in the FDA's Orange Book. Specifically, the applicant must certify that: (i) the required patent information has not been filed; (ii) the listed patent has expired; (iii) the listed patent has not expired, but will expire on a particular date and approval is sought after patent expiration; or (iv) the listed patent is invalid or will not be infringed by the new product. The ANDA applicant may also elect to submit a statement certifying that its proposed ANDA label does not contain (or carves out) any language regarding the patented method-of-use rather than certify to a listed method-of-use patent. If the applicant does not challenge the listed patents, the ANDA application will not be approved until all the listed patents claiming the referenced product have expired.

A certification that the new product will not infringe the already approved product's listed patents, or that such patents are invalid, is called a Paragraph IV certification. If the ANDA applicant has provided a Paragraph IV certification to the FDA, the applicant must also send notice of the Paragraph IV certification to the NDA and patent holders once the ANDA has been accepted for filing by the FDA. The NDA and patent holders may then initiate a patent infringement lawsuit in response to the notice of the Paragraph IV certification. The filing of a patent infringement lawsuit within 45 days of the receipt of a Paragraph IV certification automatically prevents the FDA from approving the ANDA until the earlier of 30 months, expiration of the patent, settlement of the lawsuit, or a decision in the infringement case that is favorable to the ANDA applicant.

The ANDA application also will not be approved until any applicable non-patent exclusivity listed in the Orange Book for the referenced product has expired.

Exclusivity

Upon NDA approval of a new chemical entity, or NCE, which is a drug that contains no active moiety that has been approved by the FDA in any other NDA, that drug receives five years of marketing exclusivity during which the FDA cannot receive any ANDA seeking approval of a generic version of that drug. Certain changes to a drug, such as the addition of a new indication to the package insert, are associated with a three-year period of exclusivity during which the FDA cannot approve an ANDA for a generic drug that includes the change.

An ANDA may be submitted one year before NCE exclusivity expires if a Paragraph IV certification is filed. If there is no listed patent in the Orange Book, there may not be a Paragraph IV certification, and, thus, no ANDA may be filed before the expiration of the exclusivity period.

Patent Term Extension

After NDA approval, owners of relevant drug patents may apply for up to a five year patent extension. The allowable patent term extension is calculated as half of the drug's testing phase—the time between IND application and NDA submission—and all of the review phase—the time between NDA submission and approval up to a maximum of five years. The time can be shortened if the FDA determines that the applicant did not pursue approval with due diligence. The total patent term after the extension may not exceed 14 years.

The Foreign Corrupt Practices Act

The Foreign Corrupt Practices Act, or FCPA, prohibits any U.S. individual or business from paying, offering, or authorizing payment or offering of anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with accounting provisions requiring the company to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations.

Europe and Other International Government Regulation

In addition to regulations in the United States, we will be subject to a variety of regulations in other jurisdictions governing, among other things, clinical trials and any commercial sales and distribution of our products. Whether or not we obtain FDA approval for a product, we must obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical trials or marketing of the product in those countries. Some countries outside of the United States have a similar process that requires the submission of a clinical trial application, or CTA, much like the IND prior to the commencement of human clinical trials. In Europe, for example, a CTA must be submitted to each country's national health authority and an independent ethics committee, much like the FDA and IRB, respectively. Once the CTA is approved in accordance with a country's requirements, clinical trial development may proceed.

To obtain regulatory approval to commercialize a new drug under European Union regulatory systems, we must submit a marketing authorization application, or MAA. The MAA is similar to the NDA, with the exception of, among other things, country-specific document requirements.

For other countries outside of the European Union, such as countries in Eastern Europe, Latin America or Asia, the requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. In all cases, again, the clinical trials are conducted in accordance with GCP and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

Compliance

During all phases of development (pre- and post-marketing), failure to comply with applicable regulatory requirements may result in administrative or judicial sanctions. These sanctions could include the FDA's imposition of a clinical hold on trials, refusal to approve pending applications, withdrawal of an approval, warning letters, product recalls, product seizures, total or partial suspension of production or distribution, product detention or refusal to permit the import or export of products, injunctions, fines, civil penalties or criminal prosecution. Any agency or judicial enforcement action could have a material adverse effect on us.

Other Special Regulatory Procedures

Orphan Drug Designation

The FDA may grant Orphan Drug Designation to drugs intended to treat a rare disease or condition that affects fewer than 200,000 individuals in the United States, or, if the disease or condition affects more than 200,000 individuals in the United States, there is no reasonable expectation that the cost of developing and making the drug would be recovered from sales in the United States. In the European Union, the EMA's Committee for Orphan Medicinal Products, or COMP, grants Orphan Drug Designation to promote the development of products that are intended for the diagnosis,

prevention or treatment of life-threatening or chronically debilitating conditions affecting not more than five in 10,000 persons in the European Union community. Additionally, designation is granted for products intended for the diagnosis, prevention or treatment of a life-threatening, seriously debilitating or serious and chronic condition and when, without incentives, it is unlikely that sales of the drug in the European Union would be sufficient to justify the necessary investment in developing the drug.

In the United States, Orphan Drug Designation entitles a party to financial incentives, such as opportunities for grant funding towards clinical trial costs, tax credits for certain research and user fee waivers under certain circumstances. In addition, if a product receives the first FDA approval for the indication for which it has orphan designation, the product is entitled to seven years of market exclusivity, which means the FDA may not approve any other application for the same drug for the same indication for a period of seven years, except in limited circumstances, such as a showing of clinical superiority over the product with orphan exclusivity. Orphan drug exclusivity does not prevent the FDA from approving a different drug for the same disease or condition, or the same drug for a different disease or condition.

In the European Union, Orphan Drug Designation also entitles a party to financial incentives such as reduction of fees or fee waivers and ten years of market exclusivity is granted following drug approval. This period may be reduced to six years if the Orphan Drug Designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity.

Orphan drug designation must be requested before submission of an application for marketing approval. Orphan drug designation does not convey any advantage in, or shorten the duration of the regulatory review and approval process.

Priority Review (United States) and Accelerated Review (European Union)

Based on results of the Phase 3 clinical trial(s) submitted in an NDA, upon the request of an applicant, a priority review designation may be granted to a product by the FDA, which sets the target date for FDA action on the application at six months from FDA filing, or eight months from the sponsor's submission. Priority review is given where preliminary estimates indicate that a product, if approved, has the potential to provide a safe and effective therapy where no satisfactory alternative therapy exists, or a significant improvement compared to marketed products is possible. If criteria are not met for priority review, the standard FDA review period is ten months from FDA filing, or 12 months from sponsor submission. Priority review designation does not change the scientific/medical standard for approval or the quality of evidence necessary to support approval.

Under the Centralized Procedure in the European Union, the maximum timeframe for the evaluation of a marketing authorization application is 210 days (excluding clock stops, when additional written or oral information is to be provided by the applicant in response to questions asked by the CHMP). Accelerated evaluation might be granted by the CHMP in exceptional cases, when a medicinal product is expected to be of a major public health interest, defined by three cumulative criteria: the seriousness of the disease (e.g., heavy disabling or life-threatening diseases) to be treated; the absence or insufficiency of an appropriate alternative therapeutic approach; and anticipation of high therapeutic benefit. In this circumstance, EMA ensures that the opinion of the CHMP is given within 150 days.

Pediatric Information

Under the Pediatric Research Equity Act, or PREA, NDAs or supplements to NDAs must contain data to assess the safety and effectiveness of the drug for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the drug is safe and effective. The FDA may grant full or partial waivers, or deferrals, for

submission of data. Unless otherwise required by regulation, PREA does not apply to any drug for an indication for which orphan designation has been granted.

The Best Pharmaceuticals for Children Act, or BPCA, provides NDA holders a six-month extension of any exclusivity—patent or non-patent—for a drug if certain conditions are met. Conditions for exclusivity include the FDA's determination that information relating to the use of a new drug in the pediatric population may produce health benefits in that population, the FDA making a written request for pediatric studies, and the applicant agreeing to perform, and reporting on, the requested studies within the statutory timeframe. Applications under the BPCA are treated as priority applications, with all of the benefits that designation confers.

Healthcare Reform

In March 2010, the President of the United States signed one of the most significant healthcare reform measures in decades. The Affordable Care Act, substantially changes the way healthcare will be financed by both governmental and private insurers, and significantly impacts the pharmaceutical industry. The Affordable Care Act will impact existing government healthcare programs and will result in the development of new programs. For example, the Affordable Care Act provides for Medicare payment for performance initiatives and improvements to the physician quality reporting system and feedback program.

Among the Affordable Care Act's provisions of importance to the pharmaceutical industry are 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 some government healthcare programs, beginning in 2011;
- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program, retroactive to January 1, 2010, to 23% and 13% 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 the manufacturers' outpatient drugs to be covered under Medicare Part D;
- extension of manufacturers' 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 beginning in 2014 and by adding new mandatory eligibility categories for individuals with income at or below 133% of the Federal Poverty Level, thereby potentially increasing manufacturers' Medicaid rebate liability;
- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;
- new requirements to report annually specified financial arrangements with physicians and teaching hospitals, as defined in the Affordable Care Act and its implementing regulations, including reporting any "payments or transfers of value" made or distributed to prescribers, teaching hospitals, and other healthcare providers and reporting any ownership and investment

interests held by physicians and other healthcare providers and their immediate family members and applicable group purchasing organizations during the preceding calendar year, with data collection to be required beginning August 1, 2013 and reporting to the Centers for Medicare and Medicaid Services to be required by March 31, 2014 and by the 90th day of each subsequent calendar year;

- 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.

The Affordable Care Act also establishes an Independent Payment Advisory Board, or IPAB, to reduce the per capita rate of growth in Medicare spending. Beginning in 2014, IPAB is mandated to propose changes in Medicare payments if it determines that the rate of growth of Medicare expenditures exceeds target growth rates. The IPAB has broad discretion to propose policies to reduce expenditures, which may have a negative impact on payment rates for pharmaceutical products. A proposal made by the IPAB is required to be implemented by the U.S. government's Centers for Medicare & Medicaid Services unless Congress adopts a proposal with savings greater than those proposed by the IPAB. IPAB proposals may impact payments for physician and free-standing services beginning in 2015 and for hospital services beginning in 2020.

In addition, other legislative changes have been proposed and adopted since the Affordable Care Act was enacted. On August 2, 2011, the President signed into law the Budget Control Act of 2011, which, among other things, created the Joint Select Committee on Deficit Reduction to recommend proposals in spending reductions to Congress. The Joint Select Committee did not achieve its targeted deficit reduction of at least \$1.2 trillion for the years 2013 through 2021, triggering the legislation's automatic reductions to several government programs. These reductions include aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, starting in 2013. On January 2, 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding, which could have a material adverse effect on our customers and accordingly, our financial operations.

We anticipate that the Affordable Care Act will result in additional downward pressure on coverage and the price that we receive for any approved product, and could seriously harm our business. 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. In addition, it is possible that there will be further legislation or regulation that could harm our business, financial condition, and results of operations.

Coverage and Reimbursement

In the US, many independent third-party payers, as well as the Medicare and state Medicaid programs, reimburse buyers of pharmaceutical products. Medicare is the federal program that provides health care benefits to senior citizens and certain disabled and chronically ill persons. Medicaid is the federal program administered by the states to provide health care benefits to certain indigent persons. In return for including our pharmaceutical commercial products in the Medicare and Medicaid programs, we may need to agree to pay a rebate to state Medicaid agencies that provide reimbursement for those products. We will also have to agree to sell our commercial products under contracts with the Department of Veterans Affairs, Department of Defense, Public Health Service, and numerous other federal agencies as well as certain hospitals that are designated as 340B covered

entities (entities designated by federal programs to receive drugs at discounted prices) at prices that are significantly below the price we may charge to commercial pharmaceutical distributors. These programs and contracts are highly regulated and may impose restrictions on our business. Failure to comply with these regulations and restrictions could result in a loss of our ability to continue receiving reimbursement for our drugs once approved.

Different pricing and reimbursement schemes exist in other countries. In the European Community, governments influence the price of pharmaceutical products through their pricing and reimbursement rules and control of national health care systems that fund a large part of the cost of those products to consumers. Some jurisdictions operate positive and negative list systems under which products may only be marketed once a reimbursement price has been agreed. To obtain reimbursement or pricing approval, some of these countries may require the completion of clinical trials that compare the cost-effectiveness of a particular drug candidate to currently available therapies. Other member states allow companies to fix their own prices for medicines, but monitor and control company profits. The downward pressure on health care costs in general, particularly prescription drugs, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products. In addition, in some countries, cross-border imports from low-priced markets exert a commercial pressure on pricing within a country. There can be no assurance that any country that has price controls or reimbursement limitations for drug products will allow favorable reimbursement and pricing arrangements of our products.

Other Healthcare Laws and Compliance Requirements

The federal Anti-Kickback Statute prohibits, among other things, knowingly and willfully offering, paying, soliciting or receiving remuneration to induce or in return for purchasing, leasing, ordering or arranging for the purchase, lease or order of any healthcare item or service reimbursable under Medicare, Medicaid or other federally financed healthcare programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on one hand and prescribers, purchasers, and formulary managers on the other. Although there are a number of statutory exemptions and regulatory safe harbors protecting some business arrangements from prosecution, the exemptions and safe harbors are drawn narrowly and practices that involve remuneration intended to induce prescribing, purchasing or recommending may be subject to scrutiny if they do not qualify for an exemption or safe harbor. Our practices may not in all cases meet all of the criteria for safe harbor protection from federal Anti-Kickback Statute liability. The reach of the Anti-Kickback Statute was broadened by the Affordable Care Act, which, among other things, amends the intent requirement of the federal Anti-Kickback Statute. Pursuant to the statutory amendment, 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. In addition, the Affordable Care Act 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 civil False Claims Act (discussed below) or the civil monetary penalties statute, which imposes penalties against any person who is determined to have presented or caused to be presented a claim to a federal health program that the person knows or should know is for an item or service that was not provided as claimed or is false or fraudulent.

The federal False Claims Act prohibits any person from knowingly presenting, or causing to be presented, a false claim for payment to the federal government or knowingly making, using, or causing to be made or used a false record or statement material to a false or fraudulent claim to the federal government. As a result of a modification made by the Fraud Enforcement and Recovery Act of 2009, a claim includes "any request or demand" for money or property presented to the U.S. government. Recently, several pharmaceutical and other healthcare companies have been prosecuted under these laws for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product. Other companies have been prosecuted for causing false claims

to be submitted because of the companies' marketing of the product for unapproved, and thus non-reimbursable, uses. HIPAA created new federal criminal statutes that prohibit knowingly and willfully executing a scheme to defraud any healthcare benefit program, including private third-party payors and knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services. Also, many states have similar fraud and abuse statutes or regulations that apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payor.

In addition, we may be subject to data privacy and security regulation by both the federal government and the states in which we conduct our business. HIPAA, as amended by HITECH, and its implementing regulations, imposes requirements relating to the privacy, security and transmission of individually identifiable health information. Among other things, HITECH makes HIPAA's privacy and security standards directly applicable to "business associates"—independent contractors or agents of covered entities that receive or obtain protected health information in connection with providing a service on behalf of a covered entity. HITECH also increased the civil and criminal penalties that may be imposed against covered entities, business associates and possibly other persons, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorney's fees and costs associated with pursuing federal civil actions. In addition, state laws govern the privacy and security of health information in specified circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts.

In the United States, our activities are potentially subject to additional regulation by various federal, state and local authorities in addition to the FDA, including the Centers for Medicare and Medicaid Services, other divisions of HHS (e.g., the Office of Inspector General), the DOJ and individual U.S. Attorney offices within the DOJ, and state and local governments. If a drug product is reimbursed by Medicare or Medicaid, pricing and rebate programs must comply with, as applicable, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 as well as the Medicaid rebate requirements of the Omnibus Budget Reconciliation Act of 1990, or the OBRA, and the Veterans Health Care Act of 1992, each as amended. Among other things, the OBRA requires drug manufacturers to pay rebates on prescription drugs to state Medicaid programs and empowers states to negotiate rebates on pharmaceutical prices, which may result in prices for our future products that will likely be lower than the prices we might otherwise obtain. If products are made available to authorized users of the Federal Supply Schedule of the General Services Administration, additional laws and requirements apply. Under the Veterans Health Care Act, or VHCA, drug companies are required to offer some drugs at a reduced price to a number of federal agencies including the U.S. Department of Veterans Affairs and DoD, the Public Health Service and some private Public Health Service designated entities in order to participate in other federal funding programs including Medicaid. Recent legislative changes require that discounted prices be offered for specified DoD purchases for its TRICARE program via a rebate system. Participation under the VHCA requires submission of pricing data and calculation of discounts and rebates pursuant to complex statutory formulas, as well as the entry into government procurement contracts governed by the Federal Acquisition Regulation.

Because of the breadth of these laws and the narrowness of available statutory and regulatory exemptions, it is possible that some of our business activities could be subject to challenge under one or more of such laws. If our operations are found to be in violation of any of the federal and state laws described above or any other governmental regulations that apply to us, we may be subject to penalties, including criminal and significant civil monetary penalties, damages, fines, imprisonment, exclusion from participation in government programs, injunctions, recall or seizure of products, total or partial suspension of production, denial or withdrawal of pre-marketing product approvals, private "qui tam" actions brought by individual whistleblowers in the name of the government or refusal to allow us to

enter into supply contracts, including government contracts, and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations. To the extent that any of our products are sold in a foreign country, we may be subject to similar foreign laws and regulations, which may include, for instance, applicable post-marketing requirements, including safety surveillance, anti-fraud and abuse laws, and implementation of corporate compliance programs and reporting of payments or transfers of value to healthcare professionals.

In order to distribute products commercially, we must comply with state laws that require the registration of manufacturers and wholesale distributors of pharmaceutical products in a state, including, in some states, manufacturers and distributors who ship products into the state even if such manufacturers or distributors have no place of business within the state. Some states also impose requirements on manufacturers and distributors to establish the pedigree of product in the chain of distribution, including some states that require manufacturers and others to adopt new technology capable of tracking and tracing product as it moves through the distribution chain. Several states have enacted legislation requiring pharmaceutical companies to, among other things, establish marketing compliance programs, file periodic reports with the state, make periodic public disclosures on sales, marketing, pricing, clinical trials and other activities, and/ or register their sales representatives, as well as to prohibit pharmacies and other healthcare entities from providing specified physician prescribing data to pharmaceutical companies for use in sales and marketing, and to prohibit other specified sales and marketing practices. All of our activities are potentially subject to federal and state consumer protection and unfair competition laws.

Employees

As of December 31, 2013, we had 64 employees. We have no collective bargaining agreements with our employees and have not experienced any work stoppages. We believe that relations with our employees are good.

Facilities

Our corporate headquarters and research facilities are located in Newtown, Pennsylvania, where we lease an aggregate of approximately 9,500 square feet of office and laboratory space, pursuant to lease agreements, the terms of which expire in March 2014 and September 2014, respectively. We have a second office located in Pennington, New Jersey, where we lease an aggregate of approximately 5,200 square feet of office space pursuant to lease agreements, the terms of which expire in February 2015 and October 2014, respectively. This facility houses our clinical development, clinical operations, regulatory and commercial personnel.

We believe that our existing facilities are adequate for our near-term needs. When our leases expire, we may exercise renewal options or look for additional or alternate space for our operations. We believe that suitable additional or alternative space would be available if required in the future on commercially reasonable terms.

Legal Proceedings

We are not a party to any legal proceedings.

ITEM 1A. RISK FACTORS

You should carefully consider the following risk factors together with the other information contained in this Annual Report, and in prior periodic reports filed pursuant to the Securities Exchange Act of 1934, as amended and the filing with the SEC pursuant to the Securities Act of 1933, as amended. If any of the following risks actually occur, they may materially harm our business and our financial condition and results of operations. In this event, the market price of our common stock could decline and your investment could be lost.

Risks Related to Our Financial Position and Capital Needs

We have incurred significant losses since our inception and anticipate that we will continue to incur losses in the future.

We are a clinical-stage biopharmaceutical company. Investment in biopharmaceutical product development is highly speculative because it entails substantial upfront capital expenditures and significant risk that a product candidate will fail to gain regulatory approval or become commercially viable. We do not have any products approved by regulatory authorities for marketing and have not generated any revenue from product sales to date, and we continue to incur significant research, development and other expenses related to our ongoing operations. As a result, we are not profitable and have incurred losses in every reporting period since our inception in 1998. For the years ended December 31, 2013, 2012 and 2011, we reported net losses of \$62.6 million, \$29.9 million and \$26.3 million, respectively, and we had an accumulated deficit of \$231.0 million at December 31, 2013.

We expect to continue to incur significant expenses and operating losses for the foreseeable future. We anticipate these losses to increase as we continue the research and development of, and seek regulatory approvals for, our product candidates, and potentially begin to commercialize any products that may achieve regulatory approval. We may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business. The size of our future net losses will depend, in part, on the rate of future growth of our expenses and our ability to generate revenues. If any of our product candidates fail in clinical trials or do not gain regulatory approval, or if approved, fail to achieve market acceptance, we may never become profitable. Even if we achieve profitability in the future, we may not be able to sustain profitability in subsequent periods.

We have a limited operating history, which may make it difficult for you to evaluate the success of our business to date and to assess our future viability.

Our operations to date have been limited to organizing and staffing our company, acquiring product and technology rights, discovering novel molecules and conducting product development activities for our product candidates. We have not yet obtained regulatory approval for any of our product candidates. Consequently, any predictions about our future success, performance or viability may not be as accurate as they could be if we had a longer operating history or approved products on the market.

We currently have no source of product revenue and may never become profitable.

To date, we have not generated any revenues from commercial product sales. Our ability to generate revenue from product sales and achieve profitability will depend upon our ability to successfully commercialize products, including any of our current product candidates, or other product candidates that we may in-license or acquire in the future. Even if we are able to successfully achieve regulatory approval for these product candidates, we do not know when any of these products will generate revenue from product sales for us, if at all. Our ability to generate revenue from product sales

from our current or future product candidates also depends on a number of additional factors, including our ability to:

- successfully complete development activities, including the necessary clinical trials;
- complete and submit new drug applications, or NDAs, to the U.S. Food and Drug Administration, or FDA, and obtain regulatory approval for indications for which there is a commercial market;
- complete and submit applications to, and obtain regulatory approval from, foreign regulatory authorities;
- successfully complete all required regulatory agency inspections;
- set a commercially viable price for our products;
- obtain commercial quantities of our products at acceptable cost levels;
- develop a commercial organization capable of sales, marketing and distribution for any products we intend to sell ourselves in the markets in which we choose to commercialize on our own;
- find suitable distribution partners to help us market, sell and distribute our approved products in other markets; and
- obtain coverage and adequate reimbursement from third parties, including government and private payors.

In addition, because of the numerous risks and uncertainties associated with product development, including that our product candidates may not advance through development or achieve the endpoints of applicable clinical trials, we are unable to predict the timing or amount of increased expenses, or when or if we will be able to achieve or maintain profitability. Even if we are able to complete the development and regulatory process for any product candidates, we anticipate incurring significant costs associated with commercializing these products.

Even if we are able to generate revenues from the sale of our products, we may not become profitable and may need to obtain additional funding to continue operations. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce our operations.

We are likely to require additional capital to fund our operations and if we fail to obtain necessary financing, we may be unable to complete the development and potential commercialization of our product candidates.

Our operations have consumed substantial amounts of cash since inception. We expect to continue to spend substantial amounts to advance the clinical development of our product candidates and launch and commercialize any product candidates for which we receive regulatory approval, including potentially building our own commercial organization. We believe that existing cash and cash equivalents and interest thereon, will be sufficient to fund our projected operating requirements for at least the next 12 months. However, we will likely require additional capital for the further development and potential commercialization of our product candidates and may also need to raise additional funds sooner to pursue a more accelerated development of our product candidates.

Our forecast of the period of time through which our financial resources will be adequate to support our operations is a forward-looking statement and involves risks and uncertainties, and actual results could vary as a result of a number of factors, including the factors discussed elsewhere in this "Risk Factors" section. We have based this estimate on assumptions that may prove to be wrong, and

we could utilize our available capital resources sooner than we currently expect. Our future funding requirements, both near and long-term, will depend on many factors, including, but not limited to:

- the initiation, progress, timing, costs and results of clinical trials for our product candidates and future product candidates, including our Phase 2 and Phase 3 clinical trials for rigosertib, and our Phase 1 trials for, briciclib and recilisib;
- the clinical development plans we establish for these and other product candidates;
- the achievement of milestones and our obligation to make royalty payments to Temple, LLS, or any other future product candidate licensor, if any, under our in-licensing agreements;
- the number and characteristics of product candidates that we discover or in-license and develop;
- the outcome, timing and cost of regulatory review by the FDA and comparable foreign regulatory authorities, including the potential for the FDA or comparable foreign regulatory authorities to require that we perform more studies than those that we currently expect;
- the costs of filing, prosecuting, defending and enforcing any patent claims and maintaining and enforcing other intellectual property rights;
- the effect of competing technological and market developments;
- the costs and timing of the implementation of commercial-scale outsourced manufacturing activities; and
- the costs and timing of establishing sales, marketing and distribution capabilities for any product candidates for which we may receive regulatory approval in territories where we choose to commercialize products on our own.

If we are unable to expand our operations or otherwise capitalize on our business opportunities due to a lack of capital, our ability to become profitable will be compromised.

Raising additional capital may cause dilution to our existing stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

Until we can generate substantial revenue from product sales, if ever, we expect to seek additional capital through a combination of private and public equity offerings, debt financings, strategic collaborations and alliances and licensing arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interests of existing stockholders will be diluted, and the terms may include liquidation or other preferences that adversely affect the rights of existing stockholders. Debt financing, if available, may involve agreements that include restrictive covenants limiting our ability to take important actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through strategic collaborations and alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies or product candidates, or grant licenses on terms that are not favorable to us. If we are unable to raise additional funds through equity or debt financing when needed, we may be required to delay, limit, reduce or terminate our product development or commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Unstable market and economic conditions may have serious adverse consequences on our business, financial condition and stock price.

As has been widely reported, global credit and financial markets have at times experienced disruptions over the past several years, including diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, increases in unemployment rates and uncertainty

about economic stability. There can be no assurance that deterioration in credit and financial markets and confidence in economic conditions will not occur. Our general business strategy may be compromised by economic downturns, a volatile business environment and unpredictable and unstable market conditions. If the current equity and credit markets deteriorate, it may make any necessary debt or equity financing more difficult to secure, more costly, or more dilutive. Failure to secure any necessary financing in a timely manner and on favorable terms could harm our growth strategy, financial performance and stock price and could require us to delay or abandon our business and clinical development plans. In addition, there is a risk that one or more of our current service providers, manufacturers and other partners may experience financial difficulties, which could directly affect our ability to attain our operating goals in accordance with our schedule and budget.

Risks Related to Our Business and Industry

Our future success is dependent primarily on the regulatory approval and commercialization of our product candidates, including rigosertib, which is currently undergoing Phase 2 and 3 clinical trials.

We do not have any products that have gained regulatory approval. Currently, our only clinical-stage product candidates are rigosertib, briciclib and recilisib, and rigosertib IV is our only late-stage product candidate.

As a result, our business is substantially dependent on our ability to obtain regulatory approval for, and, if approved, to successfully commercialize rigosertib and, to a lesser degree, briciclib and recilisib in a timely manner. We cannot commercialize product candidates in the United States without first obtaining regulatory approval for the product from the FDA. Similarly, we cannot commercialize product candidates outside of the United States without obtaining regulatory approval from comparable foreign regulatory authorities. Before obtaining regulatory approvals for the commercial sale of any product candidate for a target indication, we must demonstrate with substantial evidence gathered in preclinical and well-controlled clinical studies, generally including two well-controlled Phase 3 trials, and, with respect to approval in the United States, to the satisfaction of the FDA, that the product candidate is safe and effective for use for that target indication and that the manufacturing facilities, processes and controls are adequate. Even if rigosertib or another product candidate were to successfully obtain approval from the FDA and comparable foreign regulatory authorities, any approval might contain significant limitations related to use restrictions for specified age groups, warnings, precautions or contraindications, or may be subject to burdensome post-approval study or risk management requirements. If we are unable to obtain regulatory approval for rigosertib in one or more jurisdictions, or any approval contains significant limitations, we may not be able to obtain sufficient funding or generate sufficient revenue to continue the development of briciclib, recilisib, or any other product candidate that we may discover, in-license, develop or acquire in the future. Furthermore, even if we obtain regulatory approval for rigosertib, we will still need to develop a commercial organization, establish commercially viable pricing and obtain approval for adequate reimbursement from third-party and government payors. If we or our commercialization collaborators are unable to successfully commercialize rigosertib, we may not be able to earn sufficient revenues to continue our business.

The results of preclinical testing or earlier clinical studies are not necessarily predictive of future results, rigosertib, or any other product candidate we advance into clinical trials may not have favorable results in later-stage clinical trials or receive regulatory approval.

Success in preclinical testing and earlier clinical studies does not ensure that later clinical trials will generate adequate data to demonstrate the efficacy and safety of an investigational drug. A number of companies in the pharmaceutical and biotechnology industries, including those with greater resources and experience, have suffered significant setbacks in clinical trials, even after seeing promising results in earlier clinical trials. Despite the results reported in earlier clinical trials for rigosertib and our other clinical-stage product candidates, we do not know whether the later-stage clinical trials we may conduct

in the future will demonstrate adequate efficacy and safety to result in regulatory approval to market any of our product candidates in any particular jurisdiction. If later-stage clinical trials do not produce favorable results, our ability to achieve regulatory approval for any of our product candidates may be adversely impacted.

Clinical drug development involves a lengthy and expensive process with an uncertain outcome.

Clinical testing is expensive, can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical trial process. 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 early clinical trials.

We may experience delays in our ongoing or future clinical trials and we do not know whether planned clinical trials will begin or enroll subjects on time, need to be redesigned or be completed on schedule, if at all. For example, we experienced a clinical hold with our initial IND submission for recilisib based on the need to conduct additional toxicology studies and to revise quality requirements for manufacture of the drug product. While we do not anticipate any such delays, there can be no assurance that the FDA will not put clinical trials of recilisib or any other of our product candidates on clinical hold in the future. Clinical trials may be delayed, suspended or prematurely terminated for a variety of reasons, such as:

- delay or failure in reaching agreement with the FDA or a comparable foreign regulatory authority on a trial design that we are able to execute;
- delay or failure in obtaining authorization to commence a trial or inability to comply with conditions imposed by a regulatory authority regarding the scope or design of a clinical study;
- delay or failure in reaching agreement on acceptable terms with prospective contract research organizations, or CROs, and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- delay or failure in obtaining institutional review board, or IRB, approval or the approval of other reviewing entities, including comparable foreign regulatory authorities, to conduct a clinical trial at each site;
- withdrawal of clinical trial sites from our clinical trials as a result of changing standards of care or the ineligibility of a site to participate in our clinical trials;
- delay or failure in recruiting and enrolling suitable subjects to participate in a trial;
- delay or failure in subjects completing a trial or returning for post-treatment follow-up;
- clinical sites and investigators deviating from trial protocol, failing to conduct the trial in accordance with regulatory requirements, or dropping out of a trial;
- inability to identify and maintain a sufficient number of trial sites, many of which may already be engaged in other clinical trial programs, including some that may be for the same indication;
- failure of our third-party clinical trial managers to satisfy their contractual duties or meet expected deadlines;
- delay or failure in adding new clinical trial sites;
- delay or failure in meeting regulatory agency inspectional requirements;
- ambiguous or negative interim results or results that are inconsistent with earlier results;

- feedback from the FDA, the IRB, data safety monitoring boards, or a comparable foreign regulatory authority, or results from earlier stage or concurrent preclinical and clinical studies, that might require modification to the protocol for the trial;
- decision by the FDA, the IRB, a comparable foreign regulatory authority, or us, or recommendation by a data safety monitoring board or comparable foreign regulatory authority, to suspend or terminate clinical trials at any time for safety issues or for any other reason;
- unacceptable risk-benefit profile, unforeseen safety issues or adverse side effects;
- failure to demonstrate a benefit from using a drug;
- difficulties in manufacturing or obtaining from third parties sufficient quantities of a product candidate for use in clinical trials;
- lack of adequate funding to continue the clinical trial, including the incurrence of unforeseen costs due to enrollment delays, requirements to conduct additional clinical studies or increased expenses associated with the services of our CROs and other third parties; or
- changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial.

Patient enrollment, a significant factor in the timing of clinical trials, is affected by many factors including the size and nature of the patient population, the proximity of subjects to clinical sites, the eligibility criteria for the trial, the design of the clinical trial, ability to obtain and maintain patient consents, risk that enrolled subjects will drop out before completion, competing clinical trials and clinicians' and patients' perceptions as to the potential advantages of the drug being studied in relation to other available therapies, including any new drugs that may be approved for the indications we are investigating. In 2011, we suspended enrollment in our Phase 1 trial of briciclib because enrollment of patients was occurring so slowly that our inventory of briciclib clinical trial material expired. We are planning to resume Phase 1 development of briciclib in 2014 with a multi-site dose escalation study in patients with advanced solid tumors refractory to current therapies. Furthermore, we rely on CROs and clinical trial sites to ensure the proper and timely conduct of our clinical trials, and while we have agreements governing their committed activities, we have limited influence over their actual performance.

If we experience delays in the completion or termination of, any clinical trial of our product candidates, the commercial prospects of our product candidates will be harmed, and our ability to generate product revenues from any of these product candidates will be delayed. In addition, any delays in completing our clinical trials will increase our costs, slow down our product candidate development and approval process and jeopardize our ability to commence product sales and generate revenues. In addition, many of the factors that could cause a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates.

The regulatory approval processes of the FDA and comparable foreign regulatory authorities are lengthy, time consuming and inherently unpredictable, and if we are ultimately unable to obtain regulatory approval for our product candidates, our business will be substantially harmed.

The time required to obtain approval by the FDA and comparable foreign regulatory authorities is unpredictable, but typically takes many years following the commencement of preclinical studies and clinical trials and depends upon numerous factors, including the substantial discretion of the regulatory authorities. In addition, approval policies, regulations, or the type and amount of clinical data necessary to gain approval may change during the course of a product candidate's clinical development and may vary among jurisdictions. We have not obtained regulatory approval for any product candidate, and it is

possible that none of our existing product candidates or any product candidates we may discover, in-license or acquire and seek to develop in the future will ever obtain regulatory approval.

Our product candidates could fail to receive regulatory approval from the FDA or a comparable foreign regulatory authority for many reasons, including:

- disagreement over the design or implementation of our clinical trials;
- failure to demonstrate that a product candidate is safe and effective for its proposed indication;
- failure of clinical trials to meet the level of statistical significance required for approval;
- failure to demonstrate that a product candidate's clinical and other benefits outweigh its safety risks;
- disagreement over our interpretation of data from preclinical studies or clinical trials;
- delay or failure in meeting regulatory agency inspectional requirements;
- disagreement over whether to accept efficacy results from clinical trial sites outside the United States or clinical trial sites where the standard of care is potentially different from that in the United States;
- the insufficiency of data collected from clinical trials of our product candidates to support the submission and filing of an NDA or other submission or to obtain regulatory approval;
- disapproval of the manufacturing processes or facilities of third-party manufacturers with whom we contract for clinical and commercial supplies; or
- changes in the approval policies or regulations that render our preclinical and clinical data insufficient for approval.

The FDA or a comparable foreign regulatory authority may require more information, including additional preclinical or clinical data to support approval, which may delay or prevent approval and our commercialization plans, or we may decide to abandon the development program altogether. Even if we do obtain regulatory approval, our product candidates may be approved for fewer or more limited indications than we request, approval contingent on the performance of costly post-marketing clinical trials, or approval with a label that does not include the labeling claims necessary or desirable for the successful commercialization of that product candidate. In addition, the FDA may require the establishment of Risk Evaluation Mitigation Strategies, or REMS, or a comparable foreign regulatory authority may require the establishment of a similar strategy, that may restrict distribution of our products and impose burdensome implementation requirements on us. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates.

Approval by the FDA does not ensure approval by foreign regulatory authorities and approval by one or more foreign regulatory authorities does not ensure approval by regulatory authorities in other countries or by the FDA. However, a failure or delay in obtaining regulatory approval in one country may have a negative effect on the regulatory process in others. We may not be able to file for regulatory approvals and even if we file we may not receive the necessary approvals to commercialize our products in any market.

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 label, or result in significant negative consequences following any marketing approval.

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 regulatory authority. For

example, patients in our earlier-stage clinical trials of rigosertib in some cases experienced side effects, some of which were severe.

As a result of undesirable side effects or safety or toxicity issues that we may experience in our clinical trials, we may not receive approval to market any product candidates, which could prevent us from ever generating revenues or achieving profitability. Results of our trials could reveal an unacceptably high 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 or deny approval of our product candidates for any or all targeted indications. These side effects could affect patient recruitment or the ability of enrolled subjects to complete the trial or result in potential product liability claims.

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

- we may be forced to suspend marketing of such product;
- regulatory authorities may withdraw their approvals of such product;
- regulatory authorities may require additional warnings on the label that could diminish the usage or otherwise limit the commercial success of such products;
- we may be required to conduct post-market studies;
- we could be sued and held liable for harm caused to subjects or patients; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of the particular product candidate, if approved.

Even if our product candidates receive regulatory approval, they may still face future development and regulatory difficulties.

Even if we obtain regulatory approval for a product candidate, it would be subject to ongoing requirements by the FDA and comparable foreign regulatory authorities governing the manufacture, quality control, further development, labeling, packaging, storage, distribution, safety surveillance, import, export, advertising, promotion, recordkeeping and reporting of safety and other post-market information. The safety profile of any product will continue to be closely monitored by the FDA and comparable foreign regulatory authorities after approval. If the FDA or comparable foreign regulatory authorities become aware of new safety information after approval of any of our product candidates, they may require labeling changes or establishment of a REMS or similar strategy, impose significant restrictions on a product's indicated uses or marketing, or impose ongoing requirements for potentially costly post-approval studies or post-market surveillance. The label ultimately approved for rigosertib, if it achieves marketing approval, may include restrictions on use.

In addition, manufacturers of drug products and their facilities are subject to continual review and periodic inspections by the FDA and other regulatory authorities for compliance with current good manufacturing practices, or cGMP, and other regulations. If we or a regulatory agency discover previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, a regulatory agency may impose restrictions on that product, the manufacturing facility or us, including requiring recall or withdrawal of the product from the market or suspension of manufacturing. If we, our product

candidates or the manufacturing facilities for our product candidates fail to comply with applicable regulatory requirements, a regulatory agency may:

- issue warning letters or untitled letters or otherwise unacceptable inspectional findings;
- mandate modifications to promotional materials or require us to provide corrective information to healthcare practitioners;
- require us to enter into a consent decree, which can include imposition of various fines, reimbursements for inspection costs, required due dates for specific actions and penalties for noncompliance;
- seek an injunction or impose civil or criminal penalties or monetary fines;
- suspend or withdraw regulatory approval;
- suspend any ongoing clinical studies;
- refuse to approve pending applications or supplements to applications filed by us;
- suspend or impose restrictions on operations, including costly new manufacturing requirements; or
- seize or detain products, refuse to permit the import or export of products, or require us to initiate a product recall.

The occurrence of any event or penalty described above may inhibit our ability to commercialize our products and generate revenue.

Advertising and promotion of any product candidate that obtains approval in the United States will be heavily scrutinized by the FDA, the Department of Justice, or the DOJ, the Office of Inspector General of the Department of Health and Human Services, or HHS, state attorneys general, members of Congress and the public. Violations, including promotion of our products for unapproved or off-label uses, are subject to enforcement letters, inquiries and investigations, and civil and criminal sanctions by the FDA. Additionally, advertising and promotion of any product candidate that obtains approval outside of the United States will be heavily scrutinized by comparable foreign regulatory authorities.

In the United States, engaging in impermissible promotion of our products for off-label uses can also subject us to false claims litigation under federal and state statutes, which can lead to civil and criminal penalties and fines and agreements that materially restrict the manner in which we promote or distribute our drug products. These false claims statutes include the federal False Claims Act, which allows any individual to bring a lawsuit against a pharmaceutical company on behalf of the federal government alleging submission of false or fraudulent claims, or causing to present such false or fraudulent claims, for payment by a federal program such as Medicare or Medicaid. If the government prevails in the lawsuit, the individual will share in any fines or settlement funds. Since 2004, these False Claims Act lawsuits against pharmaceutical companies have increased significantly in volume and breadth, leading to several substantial civil and criminal settlements based on certain sales practices promoting off-label drug uses. This growth in litigation has increased the risk that a pharmaceutical company will have to defend a false claim action, pay settlement fines or restitution, agree to comply with burdensome reporting and compliance obligations, and be excluded from the Medicare, Medicaid and other federal and state healthcare programs. If we do not lawfully promote our approved products, we may become subject to such litigation and, if we are not successful in defending against such actions, those actions could compromise our ability to become profitable.

Failure to obtain regulatory approval in international jurisdictions would prevent our product candidates from being marketed abroad.

In order to market and sell our products in the European Union and many other jurisdictions, including Japan and Korea, we must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ substantially from that required to obtain FDA approval. The regulatory approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, it is required that the product be approved for reimbursement before the product can be approved for sale in that country. We may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. We may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our products in any market. If we are unable to obtain approval of any of our product candidates by regulatory authorities in the European Union, Japan, Korea or another country, the commercial prospects of that product candidate may be significantly diminished and our business prospects could decline.

Recently enacted and future legislation, including potentially unfavorable pricing regulations or other healthcare reform initiatives, may increase the difficulty and cost for us to obtain marketing approval of and commercialize our product candidates and affect the prices we may obtain.

The regulations that govern, among other things, marketing approvals, coverage, pricing and reimbursement for new drug products vary widely from country to country. In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability to successfully sell any product candidates for which we obtain marketing approval.

In the United States, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or Medicare Modernization Act, changed the way Medicare covers and pays for pharmaceutical products. The legislation expanded Medicare coverage for drug purchases by the elderly and introduced a new reimbursement methodology based on average sales prices for physician administered drugs. In recent years, Congress has considered further reductions in Medicare reimbursement for drugs administered by physicians. The Centers for Medicare and Medicaid Services, the agency that runs the Medicare program, also has the authority to revise reimbursement rates and to implement coverage restrictions for some drugs. Cost reduction initiatives and changes in coverage implemented through legislation or regulation could decrease utilization of and reimbursement for any approved products, which in turn would affect the price we can receive for those products. While the Medicare Modernization Act and Medicare regulations apply only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates. Therefore, any reduction in reimbursement that results from federal legislation or regulation may result in a similar reduction in payments from private payors.

In March 2010, President Obama signed into law the Patient Protection and Affordable Care Act and the Health Care and Education Affordability Reconciliation Act of 2010, or the Affordable Care Act, a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for healthcare and health insurance industries, impose new taxes and fees on pharmaceutical and medical device manufacturers and impose additional health policy reforms. The Affordable Care Act expanded manufacturers' rebate liability to include covered drugs dispensed to individuals who are enrolled in

Medicaid managed care organizations, increased the minimum rebate due for innovator drugs from 15.1% of average manufacturer price, or AMP, to 23.1% of AMP and capped the total rebate amount for innovator drugs at 100% of AMP. The Affordable Care Act and subsequent legislation also changed the definition of AMP. Furthermore, the Affordable Care Act imposes a significant annual, nondeductible fee on companies that manufacture or import certain branded prescription drug products. Substantial new provisions affecting compliance have also been enacted, which may affect our business practices with healthcare practitioners, and a significant number of provisions are not yet, or have only recently become, effective. Although it is too early to determine the effect of the Affordable Care Act, it appears likely to continue the pressure on pharmaceutical pricing, especially under the Medicare program, and may also increase our regulatory burdens and operating costs.

In addition, other legislative changes have been proposed and adopted since the Affordable Care Act was enacted. More recently, on August 2, 2011, the President signed into law the Budget Control Act of 2011, which, among other things, creates the Joint Select Committee on Deficit Reduction to recommend to Congress proposals in spending reductions. The Joint Select Committee did not achieve a targeted deficit reduction of at least \$1.2 trillion for the years 2013 through 2021, triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, starting in 2013. On January 2, 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. 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. Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We cannot be sure whether additional legislative changes will be enacted, or whether the FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our product candidates, if any, may be.

In the United States, the European Union and other potentially significant markets for our product candidates, government authorities and third-party payors are increasingly attempting to limit or regulate the price of medical products and services, particularly for new and innovative products and therapies, which has resulted in lower average selling prices. Furthermore, the increased emphasis on managed healthcare in the United States and on country and regional pricing and reimbursement controls in the European Union will put additional pressure on product pricing, reimbursement and usage, which may adversely affect our future product sales and results of operations. These pressures can arise from rules and practices of managed care groups, judicial decisions and governmental laws and regulations related to Medicare, Medicaid and healthcare reform, pharmaceutical reimbursement policies and pricing in general.

Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a product candidate in a particular country, but then be subject to price regulations that delay our commercial launch of the product, possibly for lengthy time periods, which could negatively impact the revenues we are able to generate from the sale of the product in that particular country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates even if our product candidates obtain marketing approval.

Laws and regulations governing international operations may preclude us from developing, manufacturing and selling certain product candidates outside of the United States and require us to develop and implement costly compliance programs.

As we expand our operations outside of the United States, we must comply with numerous laws and regulations in each jurisdiction in which we plan to operate. The creation and implementation of international business practices compliance programs is costly and such programs are difficult to enforce, particularly where reliance on third parties is required.

The Foreign Corrupt Practices Act, or FCPA, prohibits any U.S. individual or business from paying, offering, authorizing payment or offering anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with certain accounting provisions requiring the company to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations. The anti-bribery provisions of the FCPA are enforced primarily by the DOJ. The Securities and Exchange Commission, or the SEC, is involved with enforcement of the books and records provisions of the FCPA.

Compliance with the FCPA is expensive and difficult, particularly in countries in which corruption is a recognized problem. In addition, the FCPA presents particular challenges in the pharmaceutical industry, because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals in connection with clinical studies and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions.

Various laws, regulations and executive orders also restrict the use and dissemination outside of the United States, or the sharing with certain non-U.S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. Our expanding presence outside of the United States will require us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing, or selling certain products and product candidates outside of the United States, which could limit our growth potential and increase our development costs.

The failure to comply with laws governing international business practices may result in substantial penalties, including suspension or debarment from government contracting. Violation of the FCPA can result in significant civil and criminal penalties. Indictment alone under the FCPA can lead to suspension of the right to do business with the U.S. government until the pending claims are resolved. Conviction of a violation of the FCPA can result in long-term disqualification as a government contractor. The termination of a government contract or relationship as a result of our failure to satisfy any of our obligations under laws governing international business practices would have a negative impact on our operations and harm our reputation and ability to procure government contracts. The SEC also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions.

Even if we are able to commercialize our product candidates, the products may not receive coverage and adequate reimbursement from third-party payors, which could harm our business.

Our ability to commercialize any products successfully will depend, in part, on the extent to which coverage and adequate reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers and other organizations. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, determine which medications they will cover and establish reimbursement levels. A

primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. Third-party payors may also seek additional clinical evidence, beyond the data required to obtain marketing approval, demonstrating clinical benefits and value in specific patient populations before covering our products for those patients. We cannot be sure that coverage and adequate reimbursement will be available for any product that we commercialize and, if reimbursement is available, what the level of reimbursement will be. Coverage and reimbursement may impact the demand for, or the price of, any product candidate for which we obtain marketing approval. If reimbursement is not available or is available only at limited levels, we may not be able to successfully commercialize any product candidate for which we obtain marketing approval.

There may be significant delays in obtaining coverage and reimbursement for newly approved drugs, and coverage may be more limited than the purposes for which the drug is approved by the FDA or comparable foreign regulatory authorities. Moreover, eligibility for coverage and reimbursement does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution. Interim reimbursement levels for new drugs, if applicable, may also not be sufficient to cover our costs and may only be temporary. Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost drugs and may be incorporated into existing payments for other services. Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement policies. Our inability to obtain coverage and profitable reimbursement rates from both government-funded and private payors for any approved products that we develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell our product candidates, we may be unable to generate any revenue.

We do not currently have an organization for the sale, marketing and distribution of pharmaceutical products and the cost of establishing and maintaining such an organization may exceed the cost-effectiveness of doing so. In order to market any products that may be approved by the FDA and comparable foreign regulatory authorities, we must build our sales, marketing, managerial and other non-technical capabilities or make arrangements with third parties to perform these services. If we are unable to establish adequate sales, marketing and distribution capabilities, whether independently or with third parties, we may not be able to generate product revenue and may not become profitable. We will be competing with many companies that currently have extensive and well-funded sales and marketing operations. Without an internal commercial organization or the support of a third party to perform sales and marketing functions, we may be unable to compete successfully against these more established companies.

Our commercial success depends upon attaining significant market acceptance of our product candidates, if approved, among physicians, patients, healthcare payors and the major operators of cancer clinics.

Even if we obtain regulatory approval for any of our product candidates that we may develop or acquire in the future, the product may not gain market acceptance among physicians, healthcare payors,

patients or the medical community. Market acceptance of any of our product candidates for which we receive approval depends on a number of factors, including:

- the efficacy and safety of such product candidates as demonstrated in clinical trials;
- the clinical indications for which the product candidate is approved;
- acceptance of such product candidates as a safe and effective treatment by physicians, major operators of cancer clinics and patients;
- the potential and perceived advantages of product candidates over alternative treatments;
- the safety of product candidates seen in broader patient groups, including its use outside the approved indications;
- the prevalence and severity of any side effects;
- product labeling or product insert requirements of the FDA or other regulatory authorities;
- the timing of market introduction of our products as well as competitive products;
- the cost of treatment in relation to alternative treatments;
- the availability of coverage and adequate reimbursement and pricing by third-party payors and government authorities;
- relative convenience and ease of administration; and
- the effectiveness of our sales and marketing efforts and those of our collaborators.

If any of our product candidates are approved but fail to achieve market acceptance among physicians, patients, or healthcare payors, we may not be able to generate significant revenues, which would compromise our ability to become profitable.

Our relationships with customers and third-party payors will be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

Healthcare providers, physicians and third-party payors will all play important roles in the recommendation and prescription of any product candidates for which we obtain marketing approval. Our future arrangements with third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we would market, sell and distribute our products. 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, federal and state healthcare laws and regulations pertaining to fraud and abuse and patients' rights are and will be applicable to our business. Restrictions under applicable federal and state healthcare laws and regulations that may affect our ability to operate include the following:

- the federal healthcare Anti-Kickback Statute will constrain our marketing practices, educational programs, pricing policies, and relationships with healthcare providers or other entities, by prohibiting, 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 a federal healthcare program such as Medicare and Medicaid;
- federal civil and criminal false claims laws and civil monetary penalty laws impose criminal and civil penalties, including through 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, imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program and also created federal criminal laws that prohibit knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statements in connection with the delivery of or payment for healthcare benefits, items or services;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, also imposes obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;
- the federal physician sunshine requirements under the Affordable Care Act requires manufacturers of drugs, devices, biologics and medical supplies to report annually to HHS information related to payments and other transfers of value to physicians, other healthcare providers, and teaching hospitals, and ownership and investment interests held by physicians and other healthcare providers and their immediate family members and applicable group purchasing organizations; and
- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers; some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government and may 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 govern the privacy and security of health information in specified circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, imprisonment, exclusion from government funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations. If any physicians or other healthcare providers or entities with whom we expect to do business are found to not be in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs.

Our employees may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements, which could cause significant liability for us and harm our reputation.

We are exposed to the risk of employee fraud or other misconduct, including intentional failures to comply with FDA regulations or similar regulations of comparable foreign regulatory authorities, provide accurate information to the FDA or comparable foreign regulatory authorities, comply with manufacturing standards we have established, comply with federal and state healthcare fraud and abuse laws and regulations and similar laws and regulations established and enforced by comparable foreign

regulatory authorities, report financial information or data accurately or disclose unauthorized activities to us. Employee misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. We have adopted a code of conduct for our directors, officers and employees, or the Code of Conduct, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business and results of operations, including the imposition of significant fines or other sanctions.

We face substantial competition, which may result in others discovering, developing or commercializing products before or more successfully than we do.

The development and commercialization of new drug products is highly competitive. We face competition with respect to our current product candidates, rigosertib, briciclib and recilisib, and will face competition with respect to any product candidates that we may seek to develop or commercialize in the future, from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. There are a number of large pharmaceutical and biotechnology companies that currently market and sell products or are pursuing the development of products for the treatment of the disease indications for which we are developing our product candidates. Some of these competitive products and therapies are based on scientific approaches that are the same as or similar to our approach, and others are based on entirely different approaches. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization.

Our product candidates are being developed for cancer therapeutics and radiation protection. There are a variety of available therapies and supportive care products marketed for cancer patients. In many cases, these drugs are administered in combination to enhance efficacy or to reduce side effects. Some of these drugs are branded and subject to patent protection, and others are available on a generic basis. Many of these approved drugs are well established therapies or products and are widely accepted by physicians, patients and third-party payors. Insurers and other third-party payors may also encourage the use of generic products. This may make it difficult for us to achieve market acceptance at desired levels in a timely manner to ensure viability of our business.

More established companies may have a competitive advantage over us due to their greater size, cash flows and institutional experience. Compared to us, many of our competitors may have significantly greater financial, technical and human resources.

As a result of these factors, our competitors may obtain regulatory approval of their products before we are able to obtain patent protection or other intellectual property rights which will limit our ability to develop or commercialize our product candidates. Our competitors may also develop drugs that are safer, more effective, more widely used and cheaper than ours, and may also be more successful than us in manufacturing and marketing their products. These appreciable advantages could render our product candidates obsolete or non-competitive before we can recover the expenses of development and commercialization.

Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller and other early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting

and retaining qualified scientific, management and commercial personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

If we breach the license agreements or fail to negotiate new agreements pertaining to our product candidates, we could lose the ability to continue the development and potential commercialization of these product candidates.

In January 1999, we entered into an agreement with Temple, as subsequently amended, to obtain an exclusive, world-wide license to make, have made, use, sell, offer for sale and import several classes of novel compounds, including all three of our clinical-stage product candidates. In May 2010, we entered into an agreement with Mount Sinai School of Medicine, as subsequently amended, giving us the option to exclusively negotiate licenses related to certain compounds. If we fail to meet our obligations under these license agreements or if we fail to negotiate future license agreements, our rights under the licenses could be terminated, and upon the effective date of such termination, our right to use the licensed technology would terminate. While we would expect to exercise all rights and remedies available to us, including attempting to cure any breach by us, and otherwise seek to preserve our rights under the patents and other technology licensed to us, we may not be able to do so in a timely manner, at an acceptable cost or at all. Any uncured, material breach under the license agreement could result in our loss of exclusive rights and may lead to a complete termination of our product development and any commercialization efforts for the applicable product candidates.

Product liability lawsuits against us could cause us to incur substantial liabilities and to limit commercialization of any products that we may develop.

We face an inherent risk of product liability exposure related to the testing of our product candidates in human clinical trials and will face an even greater risk if we commercially sell any products that we may develop. Product liability claims may be brought against us by subjects enrolled in our clinical trials, patients, healthcare providers or others using, administering or selling our products. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates or products that we may develop;
- termination of clinical trial sites or entire trial programs;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend the related litigation;
- substantial monetary awards to trial subjects or patients;
- loss of revenue;
- diversion of management and scientific resources from our business operations; and
- the inability to commercialize any products that we may develop.

We currently hold \$10.0 million in product liability insurance coverage in the aggregate, which may not be adequate to cover all liabilities that we may incur. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise. We intend to expand our insurance coverage for products to include the sale of commercial products if we obtain marketing approval for our product candidates in development, but we may be unable to obtain commercially reasonable product liability insurance for

any products approved for marketing. Large judgments have been awarded in class action lawsuits based on drugs that had unanticipated side effects. A successful product liability claim or series of claims brought against us, particularly if judgments exceed our insurance coverage, could decrease our cash and adversely affect our business.

We will need to grow the size of our organization, and we may experience difficulties in managing this growth.

As of December 31, 2013, we had 64 employees. As our development and commercialization plans and strategies develop, or as a result of any future acquisitions, we will need additional managerial, operational, sales, marketing, financial and other resources. Our management, personnel and systems currently in place may not be adequate to support this future growth. Future growth would impose significant added responsibilities on members of management, including:

- managing our clinical trials effectively;
- identifying, recruiting, training, maintaining, motivating and integrating additional employees;
- managing our internal development efforts effectively while complying with our contractual obligations to licensors, licensees, contractors and other third parties;
- improving our managerial, development, operational and finance systems; and
- expanding our facilities.

As our operations expand, we will need to manage additional relationships with various strategic partners, suppliers and other third parties. Our future financial performance and our ability to commercialize our product candidates and to compete effectively will depend, in part, on our ability to manage any future growth effectively. To that end, we must be able to manage our development efforts and clinical trials effectively and hire, train and integrate additional management, administrative and sales and marketing personnel. Our failure to accomplish any of these tasks could prevent us from successfully growing our company.

Our future success depends on our ability to retain our executive officers and to attract, retain and motivate qualified personnel.

We are highly dependent upon Ramesh Kumar, Ph.D., President and Chief Executive Officer; Thomas McKearn, M.D., Ph.D., President, Research and Development; and Ajay Bansal, Chief Financial Officer. Although we have employment agreements with the persons named above, these agreements are at-will and do not prevent such persons from terminating their employment with us at any time. We do not maintain "key person" insurance for any of our executives or other employees, other than our President and Chief Executive Officer. The loss of the services of any of these persons could impede the achievement of our research, development and commercialization objectives.

If we are unable to attract and retain highly qualified employees, we may not be able to grow effectively.

Our future growth and success depend on our ability to recruit, retain, manage and motivate our employees. The loss of any member of our senior management team or the inability to hire or retain experienced management personnel could compromise our ability to execute our business plan and harm our operating results.

Because of the specialized scientific and managerial nature of our business, we rely heavily on our ability to attract and retain qualified scientific, technical and managerial personnel. The competition for qualified personnel in the pharmaceutical field is intense and as a result, we may be unable to continue to attract and retain qualified personnel necessary for the development of our business.

We may engage in future acquisitions that could disrupt our business, cause dilution to our stockholders and harm our financial condition and operating results.

While we currently have no specific plans to acquire any other businesses, we may, in the future, make acquisitions of, or investments in, companies that we believe have products or capabilities that are a strategic or commercial fit with our current product candidates and business or otherwise offer opportunities for our company. In connection with these acquisitions or investments, we may:

- issue stock that would dilute our existing stockholders' percentage of ownership;
- incur debt and assume liabilities; and
- incur amortization expenses related to intangible assets or incur large and immediate write-offs.

We may not be able to complete acquisitions on favorable terms, if at all. If we do complete an acquisition, we cannot assure you that it will ultimately strengthen our competitive position or that it will be viewed positively by customers, financial markets or investors. Furthermore, future acquisitions could pose numerous additional risks to our operations, including:

- problems integrating the purchased business, products or technologies;
- increases to our expenses;
- the failure to discover undisclosed liabilities of the acquired asset or company;
- diversion of management's attention from their day-to-day responsibilities;
- harm to our operating results or financial condition;
- entrance into markets in which we have limited or no prior experience; and
- potential loss of key employees, particularly those of the acquired entity.

We may not be able to complete any acquisitions or effectively integrate the operations, products or personnel gained through any such acquisition.

Our business and operations would suffer in the event of computer system failures.

Despite the implementation of security measures, our internal computer systems, and those of our CROs and other third parties on which we rely, are vulnerable to damage from computer viruses, unauthorized access, natural disasters, fire, terrorism, war and telecommunication and electrical failures. If such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our drug development programs. For example, the loss of clinical trial data from completed, ongoing or planned clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach results in a loss of or damage to our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development of our product candidates could be delayed.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.

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

Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of biological or hazardous materials.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

Business disruptions could seriously harm our future revenues and financial condition and increase our costs and expenses.

Our operations could be subject to earthquakes, power shortages, telecommunications failures, water shortages, floods, hurricanes, typhoons, fires, extreme weather conditions, medical epidemics and other natural or manmade disasters or business interruptions, for which we are predominantly self-insured. The occurrence of any of these business disruptions could seriously harm our operations and financial condition and increase our costs and expenses. We rely on third-party manufacturers to produce our product candidates. Our ability to obtain clinical supplies of product candidates could be disrupted if the operations of these suppliers is affected by a man-made or natural disaster or other business interruption. The ultimate impact on us, our significant suppliers and our general infrastructure of being consolidated in certain geographical areas is unknown, but our operations and financial condition could suffer in the event of a major earthquake, fire or other natural disaster.

We are relying on the FDA's "Animal Efficacy Rule" to demonstrate efficacy of recilisib, which could result in delays or failure at any stage of recilisib's development process, increase our development costs and adversely affect the commercial prospects of recilisib.

Because humans are not normally exposed to radiation and it would be unethical to expose humans to such, effectiveness of recilisib cannot be demonstrated in humans, but instead, under the FDA's "Animal Efficacy Rule," can be demonstrated, in part, by utilizing animal models. This effect has to be demonstrated in more than one animal species expected to be predictive of a response in humans, but an effect in a single animal species may be acceptable if that animal model is sufficiently well-characterized for predicting a response in humans. The animal study endpoint must be clearly related to the desired benefit in humans and the information obtained from animal studies must allow selection of an effective dose in humans. Safety may be demonstrated in human studies.

We may not be able to sufficiently demonstrate the animal correlation to the satisfaction of the FDA, as these correlates are difficult to establish and are often unclear. The FDA may decide that our data are insufficient for approval and require additional preclinical, clinical or other studies, refuse to approve recilisib, or place restrictions on our ability to commercialize recilisib. Furthermore, other countries, at this time, have not established criteria for review and approval of these types of products outside their normal review process. There is no "Animal Efficacy Rule" equivalent in countries other than the United States, and consequently there can be no assurance that we will be able to make a submission for marketing approval in foreign countries based on such animal data.

Risks Related to Our Dependence on Third Parties

We rely on third parties to conduct our preclinical and clinical trials. If these third parties do not successfully carry out their contractual duties or meet expected deadlines, we may not be able to obtain regulatory approval for or commercialize our product candidates.

We have relied upon and plan to continue to rely upon third-party CROs to monitor and manage data for our ongoing preclinical and clinical programs. We rely on these parties for execution of our preclinical and clinical trials, and we control only some aspects of their activities. Nevertheless, we are responsible for ensuring that each of our studies is conducted in accordance with the applicable protocol and legal, regulatory and scientific standards, and our reliance on the CROs does not relieve us of our regulatory responsibilities. We also rely on third parties to assist in conducting our preclinical studies in accordance with Good Laboratory Practices, or GLP, and the Animal Welfare Act requirements. We and our CROs are required to comply with federal regulations and current Good Clinical Practices, or GCP, which are international standards meant to protect the rights and health of patients that are enforced by the FDA, the Competent Authorities of the Member States of the European Economic Area, or EEA, and comparable foreign regulatory authorities for all of our products in clinical development. Regulatory authorities enforce GCP through periodic inspections of trial sponsors, principal investigators and trial sites. If we or any of our CROs fail to comply with applicable GCP, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot assure you that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical trials comply with GCP requirements. In addition, our clinical trials must be conducted with product produced under cGMP requirements. Failure to comply with these regulations may require us to repeat preclinical and clinical trials, which would delay the regulatory approval process.

Our CROs are not our employees, and except for remedies available to us under our agreements with such CROs, we cannot control whether or not they devote sufficient time and resources to our ongoing clinical, nonclinical and preclinical programs. If CROs do not successfully carry out their contractual duties or obligations or meet expected deadlines or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols, regulatory requirements or for other reasons, our clinical trials may be extended, delayed or terminated and we may not be able to obtain regulatory approval for or successfully commercialize our product candidates. As a result, our results of operations and the commercial prospects for our product candidates would be harmed, our costs could increase and our ability to generate revenues could be delayed.

Because we have relied on third parties, our internal capacity to perform these functions is limited. Outsourcing these functions involves risk that third parties may not perform to our standards, may not produce results in a timely manner or may fail to perform at all. In addition, the use of third-party service providers requires us to disclose our proprietary information to these parties, which could increase the risk that this information will be misappropriated. We currently have a small number of employees, which limits the internal resources we have available to identify and monitor our third-party providers. To the extent we are unable to identify and successfully manage the performance of third-party service providers in the future, our business may be adversely affected. 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.

If we lose our relationships with CROs, our drug development efforts could be delayed.

We rely on third-party vendors and CROs for preclinical studies and clinical trials related to our drug development efforts. Switching or adding additional CROs would involve additional cost and

requires management time and focus. Our CROs have the right to terminate their agreements with us in the event of an uncured material breach. In addition, some of our CROs have an ability to terminate their respective agreements with us if it can be reasonably demonstrated that the safety of the subjects participating in our clinical trials warrants such termination, if we make a general assignment for the benefit of our creditors or if we are liquidated. Identifying, qualifying and managing performance of third-party service providers can be difficult, time consuming and cause delays in our development programs. In addition, there is a natural transition period when a new CRO commences work and the new CRO may not provide the same type or level of services as the original provider. If any of our relationships with our third-party CROs terminate, we may not be able to enter into arrangements with alternative CROs or to do so on commercially reasonable terms.

We have limited experience manufacturing our product candidates on a large clinical or commercial scale and have no manufacturing facility. We are dependent on third-party manufacturers for the manufacture of our most advanced product candidate as well as on third parties for our supply chain, and if we experience problems with any third parties, the manufacturing of our product candidates or products could be delayed.

We do not own or operate facilities for the manufacture of our product candidates. We currently have no plans to build our own clinical or commercial scale manufacturing capabilities. We currently rely on a single source contract manufacturing organization, or CMO, for the chemical manufacture of active pharmaceutical ingredient for rigosertib, another CMO for the production of the rigosertib intravenous formulation, and a third CMO for the production of the rigosertib oral formulation for Phase 3 clinical trials. To meet our projected needs for clinical supplies to support our activities through regulatory approval and commercial manufacturing, the CMOs with whom we currently work will need to increase the scale of production. We may need to identify additional CMOs for continued production of supply for our product candidates. In addition, regulatory authorities enforce cGMP through periodic inspections of active pharmaceutical ingredient, or API and drug product manufacturing sites, quality control contract laboratories and distribution centers. If we or our CMO fail to comply with applicable cGMP, the manufacturing data generated and subsequent API lots and drug product batches in our supply chain may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional API and drug product manufacturing before approving our marketing applications. We have not yet identified alternate suppliers in the event the current CMOs we utilize are unable to scale production, or if we otherwise experience any problems with them. Although alternative third-party suppliers with the necessary manufacturing and regulatory expertise and facilities exist, it could be expensive and take a significant amount of time to arrange for alternative suppliers. If we are unable to arrange for alternative third-party manufacturing sources, or to do so on commercially reasonable terms or in a timely manner, we may not be able to complete development of our product candidates, or market or distribute them.

Reliance on third-party manufacturers entails risks to which we would not be subject if we manufactured product candidates or products ourselves, including reliance on the third party for regulatory compliance and quality assurance, the possibility of breach of the manufacturing agreement by the third party because of factors beyond our control, including a failure to synthesize and manufacture our product candidates or any products we may eventually commercialize in accordance with our specifications, and the possibility of termination or nonrenewal of the agreement by the third party, based on its own business priorities, at a time that is costly or damaging to us. In addition, the FDA and other regulatory authorities require that our product candidates and any products that we may eventually commercialize be manufactured according to cGMP and similar foreign standards. Any failure by our third-party manufacturers to comply with cGMP or failure to scale up manufacturing processes, including any failure to deliver sufficient quantities of product candidates in a timely manner, could lead to a delay in, or failure to obtain, regulatory approval of any of our product candidates. In addition, such failure could be the basis for the FDA to issue a warning letter, withdraw approvals for product candidates previously granted to us, or take other regulatory or legal action, including recall or

seizure of outside supplies of the product candidate, total or partial suspension of production, suspension of ongoing clinical trials, refusal to approve pending applications or supplemental applications, detention or product, refusal to permit the import or export of products, injunction, or imposing civil and criminal penalties.

Any significant disruption in our supplier relationships could harm our business. Any significant delay in the supply of a product candidate or its key materials for an ongoing clinical study could considerably delay completion of our clinical studies, product testing and potential regulatory approval of our product candidates. If our manufacturers or we are unable to purchase these key materials after regulatory approval has been obtained for our product candidates, the commercial launch of our product candidates would be delayed or there would be a shortage in supply, which would impair our ability to generate revenues from the sale of our product candidates.

We have entered into collaboration agreements with SymBio Pharmaceuticals Limited and Baxter Healthcare SA for rigosertib development and commercialization in certain territories and we may elect to enter into additional licensing or collaboration agreements to partner rigosertib in territories currently retained by us. Our dependence on such relationships may adversely affect our business.

Because we have limited resources, we seek to enter into, and in the past we have entered into, collaboration agreements with other pharmaceutical companies and may elect to enter into more of these agreements in the future. In July 2011, we entered into a license agreement with SymBio Pharmaceuticals Limited, or SymBio, as subsequently amended, granting an exclusive, royalty-bearing license for the development and commercialization of rigosertib in Japan and Korea. In September 2012, we entered into a development and license agreement with Baxter Healthcare SA, or Baxter, a subsidiary of Baxter International Inc., granting an exclusive, royalty-bearing license for the development and commercialization of rigosertib in Europe. In December 2012, we also entered into a collaboration agreement with GVK Biosciences Private Limited for the further development of two of our preclinical oncology programs. Any failure by our partners to perform their obligations or any decision by our partners to terminate these agreements could negatively impact our ability to successfully develop, obtain regulatory approvals for and commercialize the applicable product candidate. In addition, any termination of our collaboration agreements will terminate the funding we may receive under the relevant collaboration agreement and may impair our ability to fund further development efforts and our progress in our development programs.

Our commercialization strategy for rigosertib in territories currently retained by us may depend on our ability to enter into agreements with collaborators to obtain assistance and funding for the development and potential commercialization of rigosertib in those territories. Despite our efforts, we may be unable to secure additional collaborative licensing or other arrangements that are necessary for us to further develop and commercialize rigosertib. Supporting diligence activities conducted by potential collaborators and negotiating the financial and other terms of a collaboration agreement are long and complex processes with uncertain results. Even if we are successful in entering into one or more collaboration agreements, collaborations may involve greater uncertainty for us, as we have less control over certain aspects of our collaborative programs than we do over our proprietary development and commercialization programs. We may determine that continuing a collaboration under the terms provided is not in our best interest, and we may terminate the collaboration. Our collaborators could delay or terminate their agreements, and as a result rigosertib may never be successfully commercialized.

Further, our future collaborators may develop alternative products or pursue alternative technologies either on their own or in collaboration with others, including our competitors, and the priorities or focus of our collaborators may shift such that rigosertib receives less attention or resources than we would like, or they may be terminated altogether. Any such actions by our collaborators may adversely affect our business prospects and ability to earn revenues. In addition, we could have disputes

with our current or future collaborators, such as the interpretation of terms in our agreements. Any such disagreements could lead to delays in the development or commercialization of rigosertib or could result in time-consuming and expensive litigation or arbitration, which may not be resolved in our favor.

With respect to our programs that are currently not the subject of collaborations, we may enter into agreements with collaborators to share in the burden of conducting clinical trials, manufacturing and marketing these product candidates. In addition, our ability to develop additional proprietary compounds may depend on our ability to establish and maintain licensing arrangements or other collaborative arrangements with the holders of proprietary rights to such compounds. We may not be able to establish such arrangements on favorable terms or at all, and our future collaborative arrangements may not be successful.

Risks Related to Our Intellectual Property

If we are unable to protect our intellectual property rights, our competitive position could be harmed.

We depend on our ability to protect our proprietary technology. We rely on trade secret, patent, copyright and trademark laws, and confidentiality, licensing and other agreements with employees and third parties, all of which offer only limited protection. Our commercial success will depend in large part on our ability to obtain and maintain patent protection in the United States and other countries with respect to our proprietary technology and products. Where we have the right to do so under our license agreements, we seek to protect our proprietary position by filing patent applications in the United States and abroad related to our novel technologies and products that are important to our business. The patent positions of biotechnology and pharmaceutical companies generally are highly uncertain, involve complex legal and factual questions and have in recent years been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our patents, including those patent rights licensed to us by third parties, are highly uncertain.

The steps we have taken to protect our proprietary rights may not be adequate to preclude misappropriation of our proprietary information or infringement of our intellectual property rights, both inside and outside the United States. The rights already granted under any of our currently issued patents and those that may be granted under future issued patents may not provide us with the proprietary protection or competitive advantages we are seeking. If we are unable to obtain and maintain patent protection for our technology and products, or if the scope of the patent protection obtained is not sufficient, our competitors could develop and commercialize technology and products similar or superior to ours, and our ability to successfully commercialize our technology and products may be adversely affected.

With respect to patent rights, we do not know whether any of the pending patent applications for any of our licensed compounds will result in the issuance of patents that protect our technology or products, or if any of our issued patents will effectively prevent others from commercializing competitive technologies and products. Our pending applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such applications. Further, the examination process may require us or our licensor to narrow the claims for our pending patent applications, which may limit the scope of patent protection that may be obtained if these applications issue. Because the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, issued patents that we own or have licensed from third parties may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in the loss of patent protection, the narrowing of claims in such patents or the invalidity or unenforceability of such patents, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection for our technology and products. Protecting against the unauthorized use of our patented

technology, trademarks and other intellectual property rights is expensive, difficult and may in some cases not be possible. In some cases, it may be difficult or impossible to detect third-party infringement or misappropriation of our intellectual property rights, even in relation to issued patent claims, and proving any such infringement may be even more difficult.

We could be required to incur significant expenses to perfect our intellectual property rights, and our intellectual property rights may be inadequate to protect our competitive position.

The patent prosecution process is expensive and time-consuming, and we or our licensors may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we or our licensors will fail to identify patentable aspects of inventions made in the course of our development and commercialization activities before it is too late to obtain patent protection on them. Further, given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. We expect to seek extensions of patent terms in the United States and, if available, in other countries where we are prosecuting patents. In the United States, the Drug Price Competition and Patent Term Restoration Act of 1984 permits a patent term extension of up to five years beyond the expiration of the patent. However, the applicable authorities, including the FDA in the United States, and any equivalent regulatory authority in other countries, may not agree with our assessment of whether such extensions are available, and may refuse to grant extensions to our patents, or may grant more limited extensions than we request. If this occurs, our competitors may be able to take advantage of our investment in development and clinical trials by referencing our clinical and preclinical data and launch their product earlier than might otherwise be the case. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection. The laws of foreign countries may not protect our rights to the same extent as the laws of the United States, and these foreign laws may also be subject to change. For example, methods of treatment and manufacturing processes may not be patentable in certain jurisdictions. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing or in some cases not at all. Therefore we cannot be certain that we or our licensors were the first to make the inventions claimed in our owned or licensed patents or pending patent applications, or that we or our licensors were the first to file for patent protection of such inventions.

Recent patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents.

On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications will be prosecuted and may also affect patent litigation. In particular, under the Leahy-Smith Act, the United States transitioned in March 2013 to a "first to file" system in which the first inventor to file a patent application will be entitled to the patent. Third parties are allowed to submit prior art before the issuance of a patent by the U.S. Patent and Trademark Office, or the USPTO, and may become involved in opposition, derivation, reexamination, inter-partes review or interference proceedings challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, which could adversely affect our competitive position.

The USPTO recently developed new regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first to file provisions, did not become effective until March 16, 2013.

Accordingly, it is not clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents.

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

Periodic maintenance fees on any issued patent are due to be paid to the USPTO and foreign patent agencies in several stages over the lifetime of the patent. The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Non-compliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. If we or our licensors fail to maintain the patents and patent applications covering our product candidates, our competitive position would be adversely affected.

We may become involved in lawsuits to protect or enforce our intellectual property, which could be expensive, time consuming and unsuccessful.

Competitors may infringe our patents or misappropriate or otherwise violate our intellectual property rights. To counter infringement or unauthorized use, litigation may be necessary in the future to enforce or defend our intellectual property rights, to protect our trade secrets or to determine the validity and scope of our own intellectual property rights or the proprietary rights of others. This can be expensive and time consuming. Many of our current and potential competitors have the ability to dedicate substantially greater resources to defend their intellectual property rights than we can. Accordingly, despite our efforts, we may not be able to prevent third parties from infringing upon or misappropriating our intellectual property. Litigation could result in substantial costs and diversion of management resources. In addition, in an infringement proceeding, a court may decide that a patent owned by or licensed to us is invalid or unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation proceeding could put one or more of our patents at risk of being invalidated, held unenforceable or interpreted narrowly. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation.

Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could harm our business.

Our commercial success depends upon our ability and the ability of our collaborators to develop, manufacture, market and sell our product candidates, and to use our proprietary technologies without infringing the proprietary rights of third parties. We may become party to, or threatened with, future adversarial proceedings or litigation regarding intellectual property rights with respect to our products and technology, including interference or derivation proceedings before the USPTO. Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future. If we are found to infringe a third party's intellectual property rights, we could be required to 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. We could be forced, including by court order, to cease commercializing the infringing technology or product. In addition, in any such proceeding or litigation, we could be found liable for monetary damages. A finding of infringement could prevent us from commercializing our product candidates or force us to cease some of our business operations, which could materially harm our business. Any claims by third parties that we have misappropriated their confidential information or trade secrets could have a similar negative impact on our business.

We may be subject to claims that our employees have wrongfully used or disclosed alleged trade secrets of their former employers.

Many of our employees, including our senior management, were previously employed at other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Some of these employees, including each member of our senior management, executed proprietary rights, non-disclosure and non-competition agreements in connection with such previous employment. Although we try to ensure that our employees do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or these employees have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such employee's former employer. We are not aware of any threatened or pending claims related to these matters or concerning the agreements with our senior management, but in the future litigation may be necessary to defend against such claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

Intellectual property disputes could cause us to spend substantial resources and distract our personnel from their normal responsibilities.

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses, and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the market price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could compromise our ability to compete in the marketplace.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition to seeking patents for some of our technology and products, we also rely on trade secrets, including unpatented know-how, technology and other proprietary information, to maintain our competitive position. We seek to protect these trade secrets, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, corporate collaborators, outside scientific collaborators, CMOs, consultants, advisors and other third parties. We also generally enter into confidentiality and invention or patent assignment agreements with our

employees and consultants. Despite these efforts, any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts both within and outside the United States may be less willing or unwilling to protect trade secrets. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent such competitor from using that technology or information to compete with us, which could harm our competitive position.

Although we expect all of our employees to assign their inventions to us, and all of our employees, consultants, advisors and any third parties who have access to our proprietary know-how, information or technology to enter into confidentiality agreements, we cannot provide any assurances that all such agreements have been duly executed or that our trade secrets and other confidential proprietary information will not be disclosed or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating the trade secret. In addition, others may independently discover our trade secrets and proprietary information. For example, the FDA, as part of its Transparency Initiative, is currently considering whether to make additional information publicly available on a routine basis, including information that we may consider to be trade secrets or other proprietary information, and it is not clear at the present time how the FDA's disclosure policies may change in the future, if at all.

We may not be able to protect our intellectual property rights throughout the world.

Filing, prosecuting and defending patents on all of our product candidates throughout the world would be prohibitively expensive. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products, and may export otherwise infringing products to territories where we have patent protection, but where enforcement is not as strong as that in the United States. These products may compete with our products in jurisdictions where we do not have any issued patents and our patent claims or other intellectual property rights may not be effective or sufficient to prevent them from so competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents and other intellectual property protection, particularly those relating to biopharmaceuticals, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial cost and divert our efforts and attention from other aspects of our business.

Intellectual property rights do not necessarily address all potential threats to our competitive advantage.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations, and may not adequately protect our business, or permit us to maintain our competitive advantage. The following examples are illustrative:

- Others may be able to make compounds that are the same as or similar to our product candidates but that are not covered by the claims of the patents that we own or have exclusively licensed.
- We or our licensors or any strategic partners might not have been the first to make the inventions covered by the issued patent or pending patent application that we own or have exclusively licensed.

- We or our licensors or any strategic partners might not have been the first to file patent applications covering certain of our inventions.
- Others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our intellectual property rights.
- It is possible that our pending patent applications will not lead to issued patents.
- Issued patents that we own or have exclusively licensed may not provide us with any competitive advantages, or may be held invalid or unenforceable, as a result of legal challenges by our competitors.
- Our competitors might conduct research and development activities in the United States and other countries that provide a safe harbor from patent infringement claims for certain research and development activities, as well as in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets.
- We may not develop additional proprietary technologies that are patentable.
- The patents of others may have an adverse effect on our business.

Risks Related to Ownership of Our Common Stock

The trading market in our common stock has been extremely limited and substantially less liquid than the average trading market for a stock quoted on the NASDAQ Global Market.

Since our initial listing on the NASDAQ Global Market on July 25, 2013, the trading market in our common stock has been limited and substantially less liquid than the average trading market for companies quoted on the NASDAQ Global Market. The quotation of our common stock on the NASDAQ Global Market does not assure that a meaningful, consistent and liquid trading market currently exists. We cannot predict whether a more active market for our common stock will develop in the future. An absence of an active trading market could adversely affect our stockholders' ability to sell our common stock at current market prices in short time periods, or possibly at all. Additionally, market visibility for our common stock may be limited and such lack of visibility may have a depressive effect on the market price for our common stock. As of December 31, 2013, approximately 42% of our outstanding shares of common stock was held by our officers, directors, beneficial owners of 5% or more of our capital stock and their respective affiliates, which adversely affects the liquidity of the trading market for our common stock, in as much as federal securities laws restrict sales of our shares by these stockholders. If our affiliates continue to hold their shares of common stock, there will be limited trading volume in our common stock, which may make it more difficult for investors to sell their shares or increase the volatility of our stock price.

Our share price may be volatile, which could subject us to securities class action litigation and result in substantial losses to our stockholders.

The trading price of our common stock is highly volatile and could be subject to wide fluctuations in response to various factors, some of which are beyond our control. Since our initial listing on the NASDAQ Global Market on July 25, 2013 through December 31, 2013, the price of our common stock on the NASDAQ Global Market has ranged from \$11.31 per share to \$31.13 per share. In addition to the factors discussed in this "Risk Factors" section and elsewhere in this Annual Report, these factors include:

- results of clinical trials of our product candidates or those of our competitors;
- regulatory actions with respect to our products or our competitors' products;

- actual or anticipated changes in our growth rate relative to our competitors;
- announcements by us or our competitors of significant acquisitions, strategic partnerships, joint ventures, collaborations or capital commitments;
- the success of competitive products or technologies;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our product candidates or clinical development programs;
- the results of our efforts to in-license or acquire additional product candidates or products;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- variations in our financial results or those of companies that are perceived to be similar to us;
- fluctuations in the valuation of companies perceived by investors to be comparable to us;
- share price and volume fluctuations attributable to inconsistent trading volume levels of our shares;
- announcement or expectation of additional financing efforts;
- sales of our common stock by us, our insiders or our other stockholders;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors; and
- general economic, industry and market conditions.

In addition, the stock market in general, and pharmaceutical and biotechnology companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance. The realization of any of these risks or any of a broad range of other risks, including those described in these "Risk Factors," could have a dramatic and material adverse impact on the market price of our common stock.

We may be subject to securities litigation, which is expensive and could divert management attention.

The market price of our common stock has been and may continue to be volatile, and in the past companies that have experienced volatility in the market price of their stock have been subject to securities class action litigation. We may be the target of this type of litigation in the future. Securities litigation against us could result in substantial costs and divert our management's attention from other business concerns, which could seriously harm our business.

Our principal stockholders and management own a significant percentage of our stock and will be able to exert significant control over matters subject to stockholder approval.

Our executive officers, directors, holders of 5% or more of our capital stock and their respective affiliates together beneficially owned approximately 42% of our voting stock at December 31, 2013. These stockholders may be able to determine the outcome of all matters requiring stockholder approval. For example, these stockholders may be able to control elections of directors, amendments of

our organizational documents, or approval of any merger, sale of assets, or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may feel are in your best interest as one of our stockholders. The interests of this group of stockholders may not always coincide with your interests or the interests of other stockholders and they may act in a manner that advances their best interests and not necessarily those of other stockholders, including seeking a premium value for their common stock, and might affect the prevailing market price for our common stock.

We are an "emerging growth company" and we intend to take advantage of reduced disclosure and governance requirements applicable to emerging growth companies, which could result in our common stock being less attractive to investors.

We are an "emerging growth company," as defined in the JOBS Act, and we intend to take advantage of certain exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies including, but not limited to, not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements, and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. We cannot predict if investors will find our common stock less attractive because we will 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 may take advantage of these reporting exemptions until we are no longer an emerging growth company, which in certain circumstances could be for up to five years.

Our status as an "emerging growth company" under the JOBS Act may make it more difficult to raise capital as and when we need it.

Because of the exemptions from various reporting requirements provided to us as an "emerging growth company" we may be less attractive to investors and it may be difficult for us to raise additional capital as and when we need it. Investors may be unable to compare our business with other companies in our industry if they believe that our financial accounting is not as transparent as other companies in our industry. If we are unable to raise additional capital as and when we need it, our financial condition and results of operations may be materially and adversely affected.

If we fail to maintain an effective system of internal control over financial reporting in the future, we may not be able to accurately report our financial condition, results of operations or cash flows, which may adversely affect investor confidence in us and, as a result, the value of our common stock.

The Sarbanes-Oxley Act requires, among other things, that we maintain effective internal controls for financial reporting and disclosure controls and procedures. Commencing with our annual report on Form 10-K for the year ending December 31, 2014, we will be required, under Section 404 of the Sarbanes-Oxley Act, to furnish a report by management on, among other things, the effectiveness of our internal control over financial reporting. This assessment will need to include disclosure of any material weaknesses identified by our management in our internal control over financial reporting. A material weakness is a deficiency, or combination of deficiencies, in internal control over financial reporting that results in more than a reasonable possibility that a material misstatement of annual or interim financial statements will not be prevented or detected on a timely basis. Section 404 of the Sarbanes-Oxley Act also generally requires an attestation from our independent registered public accounting firm on the effectiveness of our internal control over financial reporting. However, for as long as we remain an "emerging growth company" as defined in the JOBS Act, we intend to utilize the provision exempting us from the requirement that our independent registered public accounting firm provide an attestation on the effectiveness of our internal control over financial reporting.

Our compliance with Section 404 will require that we incur substantial accounting expense and expend significant management efforts. We currently do not have an internal audit group, and we will need to hire additional accounting and financial staff with appropriate public company experience and technical accounting knowledge, and compile the system and process documentation necessary to perform the evaluation needed to comply with Section 404. We may not be able to complete our evaluation, testing and any required remediation in a timely fashion. During the evaluation and testing process, if we identify one or more material weaknesses in our internal control over financial reporting, we will be unable to assert that our internal control over financial reporting is effective. We cannot assure you that there will not be material weaknesses or significant deficiencies in our internal control over financial reporting in the future. Any failure to maintain internal control over financial reporting could severely inhibit our ability to accurately report our financial condition, results of operations or cash flows. If we are unable to conclude that our internal control over financial reporting is effective, or if our independent registered public accounting firm determines we have a material weakness or significant deficiency in our internal control over financial reporting once that firm begins its Section 404 audits of internal control over financial reporting, we could lose investor confidence in the accuracy and completeness of our financial reports, the market price of our common stock could decline, and we could be subject to sanctions or investigations by the NASDAQ Stock Market, the SEC or other regulatory authorities. Failure to remedy any material weakness in our internal control over financial reporting, or to implement or maintain other effective control systems required of public companies, could also restrict our future access to the capital markets.

If we are unable to successfully remediate the existing material weakness in our internal control over financial reporting, the accuracy and timing of our financial reporting may be adversely affected.

In preparing our consolidated financial statements as of and for the year ended December 31, 2012, we and our independent registered public accounting firm identified control deficiencies in the design and operation of our internal control over financial reporting that together constituted a material weakness in our internal control over financial reporting. A material weakness is a deficiency, or a combination of deficiencies, in internal control over financial reporting such that there is a reasonable possibility that a material misstatement of our financial statements will not be prevented or detected on a timely basis. The material weakness identified was that we did not have sufficient financial reporting and accounting staff with appropriate training in GAAP and SEC rules and regulations with respect to financial reporting. As such, our controls over financial reporting were not designed or operating effectively, and as a result there were adjustments required in connection with closing our books and records and preparing our 2012 consolidated financial statements.

We have discussed this material weakness with our independent registered public accounting firm and our Audit Committee. To remediate, this material weakness, we have expanded our staff by hiring a Chief Financial Officer, a Director of Financial Reporting and a Vice President of Financial Planning and Accounting, each with prior public company financial reporting experience. We have also hired additional finance and accounting personnel with appropriate training to build our financial management and reporting infrastructure. We have initiated processes to further develop and document our accounting policies and financial reporting procedures and to implement additional internal controls. Although, we believe we have made improvements in our disclosure controls and procedures, as of our December 31, 2013 financial closing process, we had not yet fully remediated the material weakness discussed above. In addition, we cannot provide assurance that we have identified all of our existing material weaknesses, or that we will not in the future have additional material weaknesses.

We have not yet remediated the material weakness described above, and the remediation measures that we have implemented and intend to implement may be insufficient to address our existing material weakness or to identify or prevent additional material weaknesses. If we fail to remediate the material weakness or to meet the demands that will be placed upon us as a public company, including the

requirements of the Sarbanes-Oxley Act, we may be unable to accurately report our financial results, or report them within the timeframes required by law or stock exchange regulations. Failure to comply with Section 404 of the Sarbanes-Oxley Act could also potentially subject us to sanctions or investigations by the SEC or other regulatory authorities. There is no assurance that we will be able to remediate the material weakness in a timely manner, or at all, or that in the future, additional material weaknesses will not exist or otherwise be discovered. If our efforts to remediate the material weakness identified are not successful, or if other material weaknesses or other deficiencies occur, our ability to accurately and timely report our financial position could be impaired, which could result in late filings of our annual and quarterly reports under the Exchange Act, restatements of our consolidated financial statements, a decline in our stock price, suspension or delisting of our common stock from the NASDAQ Global Market, and could adversely affect our reputation, results of operations and financial condition.

Our disclosure controls and procedures may not prevent or detect all errors or acts of fraud.

We are subject to the periodic reporting requirements of the Exchange Act. Our disclosure controls and procedures are designed to reasonably assure that information required to be disclosed by us in reports we file or submit under the Exchange Act is accumulated and communicated to management, recorded, processed, summarized and reported within the time periods specified in the rules and forms of the SEC. We believe that any disclosure controls and procedures or internal controls and procedures, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met.

These inherent limitations include the realities that judgments in decision-making can be faulty, and that breakdowns can occur because of simple error or mistake. Additionally, controls can be circumvented by the individual acts of some persons, by collusion of two or more people or by an unauthorized override of the controls. Accordingly, because of the inherent limitations in our control system, misstatements or insufficient disclosures due to error or fraud may occur and not be detected.

We will incur increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives.

As a public company, we are incurring and will continue to incur significant legal, accounting and other expenses that we did not incur as a private company, and these expenses may increase even more after we are no longer an "emerging growth company." We are subject to the reporting requirements of the Exchange Act, the Sarbanes-Oxley Act, the Dodd-Frank Wall Street Reform and Protection Act, as well as rules adopted, and to be adopted, by the SEC and NASDAQ Stock Market. Our management and other personnel need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations substantially increase our legal and financial compliance costs and make some activities more time-consuming and costly. In the future, we estimate that we will incur approximately \$2.0 to \$3.0 million of incremental costs per year associated with being a publicly traded company, although it is possible that our actual incremental costs will be higher than we currently estimate. For example, these rules and regulations can make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to incur substantial costs to maintain the sufficient coverage. We cannot predict or estimate the amount or timing of additional costs we may incur to respond to these requirements. The impact of these requirements could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, our board committees or as executive officers.

Because we do not anticipate paying any cash dividends on our capital stock in the foreseeable future, capital appreciation, if any, will be your sole source of gain.

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. In addition, the terms of any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future.

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 raise capital, we may sell substantial amounts of common stock or securities convertible into or exchangeable for common stock. These future issuances of common stock or common stock-related securities, together with the exercise of outstanding options and any additional shares issued in connection with acquisitions, if any, may result in material dilution to our investors. Such sales may also result in material dilution to our existing stockholders, and new investors could gain rights, preferences and privileges senior to those of holders of our common stock, including shares of common stock sold in this offering.

Pursuant to our equity incentive plans, our compensation committee is authorized to grant equity-based incentive awards to our directors, executive officers and other employees and service providers, including officers, employees and service providers of our subsidiaries and affiliates. The number of shares of our common stock available for future grant under our 2013 Equity Compensation Plan, which became effective in July 2013, was 676,236 as of December 31, 2013. Future option grants and issuances of common stock under our 2013 Equity Compensation Plan may have an adverse effect on the market price of our common stock.

We have broad discretion in the use of the net proceeds from our initial public offering and may not use them effectively.

On July 30, 2013, the Company completed its initial public offering of 5,941,667 shares of the Company's common stock, at a price of \$15.00 per share, including 775,000 shares of common stock issued upon the exercise in full by the underwriters of their option to purchase additional shares at the same price to cover over-allotments. The Company received net proceeds of \$79,811,000 from the sale, net of underwriting discounts and commissions and other estimated offering expenses. The offer and sale of all of the shares in the offering were registered under the Securities Act in accordance with the Company's final prospectus filed on July 25, 2013 with the SEC pursuant to Rule 424(b)(4) of the Securities Act.

We have invested the net proceeds from the offering in a variety of capital preservation investments, including short-term, investment grade, interest bearing instruments such as U.S. government securities and money market funds. We have broad discretion in the use of the net proceeds from our initial public offering and could spend the proceeds in ways that do not improve our results of operations or enhance the value of our stock. The failure by our management to apply these funds effectively could result in financial losses that could have a material adverse effect on our business, cause the market price of our common stock to decline and delay the development of our product candidates. Pending their use, we may invest the net proceeds from the offering in a manner that does not produce income or that loses value. If we do not invest the net proceeds from the offering in ways that enhance stockholder value, we may fail to achieve expected financial results, which could cause the price of our common stock to decline.

Some provisions of our charter documents and Delaware law may have anti-takeover effects that could discourage an acquisition of us by others, even if an acquisition would be beneficial to our stockholders and may prevent attempts by our stockholders to replace or remove our current management.

Provisions in our tenth amended and restated certificate of incorporation, or certificate of incorporation, and amended and restated bylaws, as well as provisions of Delaware law, could make it more difficult for a third party to acquire us or increase the cost of acquiring us, even if doing so would benefit our stockholders, or remove our current management. These include provisions that will:

- permit our board of directors to issue up to 5,000,000 shares of preferred stock, with any rights, preferences and privileges as they may designate;
- provide that all vacancies on our board of directors, including as a result of newly created directorships, may, except as otherwise required by law, be filled by the affirmative vote of a majority of directors then in office, even if less than a quorum;
- require that any action to be taken by our stockholders must be effected at a duly called annual or special meeting of stockholders and not be taken by written consent;
- provide that stockholders seeking to present proposals before a meeting of stockholders or to nominate candidates for election as directors at a meeting of stockholders must provide advance notice in writing, and also specify requirements as to the form and content of a stockholder's notice;
- not provide for cumulative voting rights, thereby allowing the holders of a majority of the shares of common stock entitled to vote in any election of directors to elect all of the directors standing for election; and
- provide that special meetings of our stockholders may be called only by the board of directors or by such person or persons requested by a majority of the board of directors to call such meetings.

These provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors, who are responsible for appointing the members of our management. Because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which may discourage, delay or prevent someone from acquiring us or merging with us whether or not it is desired by or beneficial to our stockholders. Under Delaware law, a corporation may not, in general, engage in a business combination with any holder of 15% or more of its capital stock unless the holder has held the stock for three years or, among other things, the board of directors has approved the transaction. Any provision of our amended and restated certificate of incorporation or amended and restated bylaws or Delaware law that has the effect of delaying or deterring a change in control could limit the opportunity for our stockholders to receive a premium for their shares of our common stock, and could also affect the price that some investors are willing to pay for our common stock.

If securities or industry analysts do not publish research or publish inaccurate or unfavorable research about our business, our stock price and trading volume could decline.

The trading market for our common stock is influenced by the research and reports that securities or industry analysts publish about us or our business. We do not have any control over these analysts. There can be no assurance that analysts will continue to cover us or provide favorable coverage. If one or more of the analysts who cover us downgrade our stock or change their opinion of our stock, our share price would likely decline. If one or more of these analysts cease coverage of our company or fail

to regularly publish reports on us, we could lose visibility in the financial markets, which could cause our share price or trading volume to decline.

ITEM 1B. UNRESOLVED STAFF COMMENTS

None.

ITEM 2. PROPERTIES

Our corporate headquarters and research facilities are located in Newtown, Pennsylvania, where we lease an aggregate of approximately 9,500 square feet of office and laboratory space, pursuant to lease agreements, the terms of which expire in March 2014 and September 2014, respectively. We have a second office located in Pennington, New Jersey, where we lease an aggregate of approximately 5,200 square feet of office space pursuant to lease agreements, the terms of which expire in February 2015 and October 2014, respectively. This facility houses our clinical development, clinical operations, regulatory and commercial personnel.

We believe that our existing facilities are adequate for our near-term needs. When our leases expire, we may exercise renewal options or look for additional or alternate space for our operations. We believe that suitable additional or alternative space would be available if required in the future on commercially reasonable terms.

ITEM 3. LEGAL PROCEEDINGS

We are not a party to any legal proceedings and we are not aware of any claims or actions pending or threatened against us. In the future, we might from time to time become involved in litigation relating to claims arising from our ordinary course of business.

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

Market Information

Our common stock began trading on the NASDAQ Global Market on July 25, 2013 under the symbol "ONTX." Prior to that time, there was no public market for our common stock. Shares sold in our initial public offering on July 24, 2013 were priced at \$15.00 per share.

The following table sets forth the high and low sales prices per share of our common stock as reported on the NASDAQ Global Market for the period indicated.

<u>Year Ended December 31, 2013</u>	<u>High</u>	<u>Low</u>
Fourth quarter	\$ 31.13	\$ 11.31
Third quarter (beginning July 25, 2013)	30.00	19.42

Stockholders

As of February 28, 2014, there were 216 holders of record for shares of our common stock. This does not reflect beneficial stockholders who held their common stock in "street" or nominee name through brokerage firms.

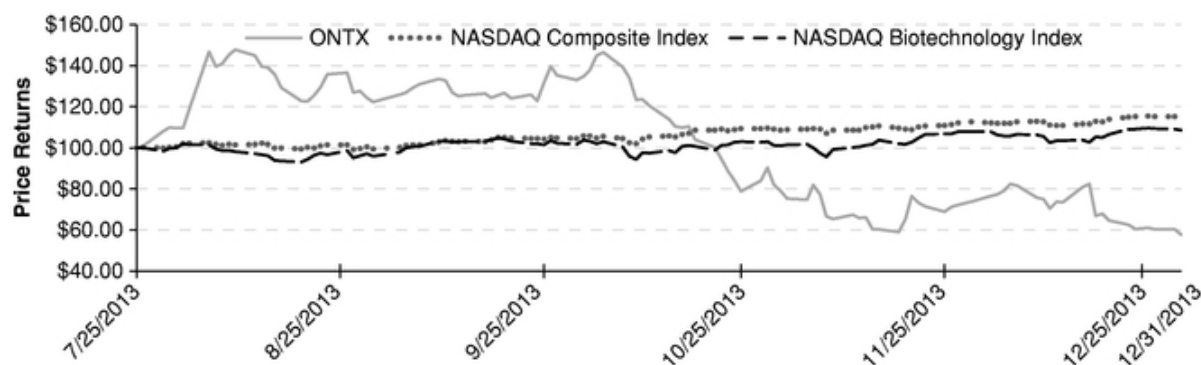
Securities Authorized for Issuance Under Equity Compensation Plans

Information regarding securities authorized for issuance under the Company's equity compensation plans is contained in Part III, Item 12 of this Annual Report.

Performance Graph

The following graph illustrates a comparison of the total cumulative stockholder return for our common stock since July 25, 2013, which is the first trading day for our stock, to two indices: the NASDAQ Composite Index and the NASDAQ Biotechnology Index. The graph assumes an initial investment of \$100 on July 25, 2013, in our common stock, the stocks comprising the NASDAQ Composite Index, and the stocks comprising the NASDAQ Biotechnology Index. Historical stockholder return is not necessarily indicative of the performance to be expected for any future periods.

Comparison of 3 Month Cumulative Total Return*
Among Onconova Therapeutics Pharmaceuticals, Inc., the NASDAQ Composite Index and the NASDAQ Biotechnology Index



* \$100 invested on 7/25/2013 in stock or index.

The performance graph shall not be deemed to be incorporated by reference by means of any general statement incorporating by reference this Annual Report into any filing under the Securities Act of 1933, as amended or the Exchange Act, except to the extent that we specifically incorporate such information by reference, and shall not otherwise be deemed filed under such acts.

Dividend Policy

We have never declared or paid any cash dividends on our capital stock. We currently intend to retain all available funds and any future earnings to support our operations and finance the growth and development of our business. We do not intend to pay cash dividends on our common stock for the foreseeable future.

Issuer Purchases of Equity Securities

We did not purchase any of our registered equity securities during the period covered by this Annual Report.

Use of Proceeds from Registered Securities

On July 30, 2013, the Company completed its initial public offering of 5,941,667 shares of the Company's common stock, at a price of \$15.00 per share, including 775,000 shares of common stock issued upon the exercise in full by the underwriters of their option to purchase additional shares at the same price to cover over-allotments. The Company received net proceeds of \$79,811,000 from the sale, net of underwriting discounts and commissions and other offering expenses. The offer and sale of all of the shares in the offering were registered under the Securities Act in accordance with the Company's final prospectus filed on July 25, 2013 with the SEC pursuant to Rule 424(b)(4) of the Securities Act.

We have invested the net proceeds from the offering in a variety of capital preservation investments, including short-term, investment grade, interest bearing instruments such as U.S. government securities and money market funds. There has been no material change in our planned use of the net proceeds from the offering as described in our final prospectus filed with the Securities and Exchange Commission pursuant to Rule 424(b)(4) under the Securities Act on July 25, 2013. We have broad discretion in the use of the net proceeds from our initial public offering and could spend the proceeds in ways that do not improve our results of operations or enhance the value of our stock.

ITEM 6. SELECTED FINANCIAL DATA

The selected financial data set forth below is derived from our audited consolidated financial statements and may not be indicative of future operating results. The following selected consolidated financial data should be read in conjunction with Item 7, "Management's Discussion and Analysis of Financial Condition and Results of Operations" and the consolidated financial statements and the notes thereto included elsewhere in this report. The selected financial data in this section are not intended to replace our consolidated financial statements and the related notes. Our historical results are not necessarily indicative of our future results.

	Years ended December 31,		
	2013	2012	2011
Consolidated Statement of Operations Data:			
Revenue	\$ 4,753,000	\$ 46,190,000	\$ 1,487,000
Operating expenses:			
General and administrative	16,793,000	15,707,000	6,436,000
Research and development	50,182,000	52,762,000	22,624,000
Total operating expenses	66,975,000	68,469,000	29,060,000
Loss from operations	(62,222,000)	(22,279,000)	(27,573,000)
Change in fair value of warrant liability	42,000	367,000	1,287,000
Interest expense	(4,000)	(8,608,000)	(19,000)
Other income, net	63,000	608,000	11,000
Net loss before income taxes	(62,121,000)	(29,912,000)	(26,294,000)
Income taxes	435,000	—	—
Net loss	(62,556,000)	(29,912,000)	(26,294,000)
Net loss attributable to non-controlling interest	13,000	—	—
Net loss attributable to Onconova Therapeutics, Inc	(62,543,000)	(29,912,000)	(26,294,000)
Accretion of redeemable convertible preferred stock	(2,320,000)	(3,953,000)	(4,020,000)
Net loss applicable to common stockholders	\$ (64,863,000)	\$ (33,865,000)	\$ (30,314,000)
Net loss per share of common stock, basic and diluted	\$ (6.12)	\$ (15.35)	\$ (14.18)
Basic and diluted weighted average shares outstanding	10,594,227	2,206,888	2,137,403

	December 31,		
	2013	2012	2011
Consolidated Balance Sheet Data:			
Cash, cash equivalents and marketable securities	\$ 100,003,000	\$ 81,527,000	\$ 2,713,000
Total assets	105,153,000	83,852,000	4,462,000
Total liabilities	24,253,000	40,843,000	12,081,000
Accumulated deficit	(230,896,000)	(168,353,000)	(138,441,000)
Total stockholders' equity (deficit)	80,900,000	(158,306,000)	(138,419,000)

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

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

Overview

We are a clinical-stage biopharmaceutical company focused on discovering and developing novel small molecule drug candidates to treat cancer. Using our proprietary chemistry platform, we have created an extensive library of targeted anti-cancer agents designed to work against specific cellular pathways that are important to cancer cells. We believe that the drug candidates in our pipeline have the potential to be efficacious in a wide variety of cancers without causing harm to normal cells. We have three clinical-stage product candidates and six preclinical programs.

Rigosertib, our most advanced product candidate, is being tested in clinical studies for myelodysplastic syndromes, or MDS and head and neck cancers. To date, we have enrolled more than 1,000 patients in rigosertib trials in hematological diseases and solid tumors. In February 2014, the Company announced top-line analysis of a Phase 3 trial of rigosertib IV in higher risk MDS patients who had progressed on, failed to respond to or relapsed after prior therapy with hypomethylating agents (HMAs). Although the results of this study showed numerical improvement in median overall survival in the rigosertib treated patients, the observed improvement of 2.4 months did not meet the required level of statistical significance. However, a statistically significant improvement in median overall survival was evident in the subset of patients who had progressed on or failed to respond to previous treatment with hypomethylating agents (HMAs). We plan to consult with regulatory authorities to determine the next steps for rigosertib IV in higher risk MDS. We are also testing rigosertib in two Phase 2 trials for transfusion-dependent lower risk MDS, in a Phase 2 trial for head and neck cancers and in a Phase 1/2 study in combination with azacitidine for first-line treatment of MDS. We also evaluated rigosertib in a Phase 3 trial for metastatic pancreatic cancer. In December 2013, a pre-planned interim futility and safety analysis for this trial was performed and as a result the study was discontinued. Baxter Healthcare SA, or Baxter, has commercialization rights for rigosertib in Europe and SymBio Pharmaceuticals Limited, or SymBio, has commercialization rights in Japan and Korea. We have retained development and commercialization rights to rigosertib in the rest of the world, including in the United States.

Our second clinical-stage product candidate is ON 013105, or *briciclib*, a small molecule targeting an important regulatory protein, cyclin D1, which is often found at elevated levels in cancer cells. We are planning to resume Phase 1 clinical development of briciclib (ON 013105) in 2014 with a multi-site dose escalation study in patients with advanced solid tumors refractory to current therapies.

Our third clinical-stage product candidate, recilisib, is being developed in collaboration with the U.S. Department of Defense for acute radiation syndromes. We have conducted animal studies and clinical trials of recilisib under the FDA's Animal Efficacy Rule, which permits marketing approval for new medical countermeasures for which human efficacy studies are not feasible or ethical, by relying on evidence from animal studies in appropriate animal models to support efficacy in humans. We have completed four Phase 1 trials to evaluate the safety and pharmacokinetics of recilisib in healthy human adult subjects using both subcutaneous and oral formulations.

In addition to our three clinical-stage product candidates, we are advancing six preclinical programs that target kinases, cellular metabolism or division. We intend to explore additional collaborations to further the development of these product candidates as we focus internally on our more advanced programs.

We were incorporated in Delaware in December 1998 and commenced operations in January 1999. Our operations to date have included our organization and staffing, business planning, raising capital, in-licensing technology from research institutions, identifying potential product candidates, developing product candidates and building strategic alliances, as well as undertaking preclinical studies and clinical trials of our product candidates.

Since commencing operations we have dedicated a significant portion of our resources to our development efforts for our clinical-stage product candidates, particularly rigosertib. We incurred research and development expenses of \$50.2 million, \$52.8 million and \$22.6 million during the years ended December 31, 2013, 2012 and 2011, respectively. We anticipate that a significant portion of our operating expenses will continue to be related to research and development as we continue to advance our preclinical programs and our clinical-stage product candidates. In July 2013, we completed our initial public offering, or IPO, from which we received net proceeds of \$79.8 million. Prior to the consummation of the IPO, we funded our operations primarily through the sale of preferred stock amounting to \$144.7 million, including \$50.0 million that Baxter invested in our Preferred Stock in 2012, as well as proceeds from the issuance of convertible debt and a stockholder loan amounting to \$26.8 million in the aggregate, all of which was later converted into shares of our Preferred Stock, and upfront payments of \$7.5 million from Symbio and \$50.0 million from Baxter in connection with our collaboration agreements. We have also received an aggregate of \$8.0 million from The Leukemia and Lymphoma Society, or LLS, under a funding agreement. Under our collaboration agreements with Baxter and Symbio, we are also eligible to receive an aggregate of up to \$359.5 million upon the achievement of specified development and regulatory milestones and up to \$280.0 million upon the achievement of specified commercialization milestones, as well as tiered royalties, at percentage rates ranging from the low-teens to low-twenties, on any future net sales of products resulting from these collaborations. As of December 31, 2013, we had \$100.0 million in cash, cash equivalents and marketable securities, respectively.

Our net losses were \$62.6 million, \$29.9 million and \$26.3 million for the years ended December 31, 2013, 2012 and 2011, respectively. We recognized revenues of \$4.8 million, \$46.2 million and \$1.5 million for the years ended December 31, 2013, 2012 and 2011, respectively. As of December 31, 2013, we had an accumulated deficit of \$230.9 million. We expect to incur significant expenses and operating losses for the foreseeable future as we continue the development and clinical trials of, and seek regulatory approval for, our product candidates, even as milestones under our license and collaboration agreements may be met. If we obtain regulatory approval for any of our product candidates, we expect to incur significant commercialization expenses. We do not currently have an organization for the sales, marketing and distribution of pharmaceutical products. We may rely on licensing and co-promotion agreements with strategic or collaborative partners for the commercialization of our products in the United States and other territories. If we choose to build a commercial infrastructure to support marketing in the United States for any of our product candidates that achieve regulatory approval, such commercial infrastructure could be expected to include a targeted, oncology sales force supported by sales management, internal sales support, an internal marketing group and distribution support. To develop the appropriate commercial infrastructure internally, we would have to invest financial and management resources, some of which would have to be deployed prior to having any certainty about marketing approval.

Furthermore, we have and expect to continue to incur additional costs associated with operating as a public company. Accordingly, we will seek to fund our operations primarily through public equity or debt financings or other sources. Other additional financing may not be available to us on acceptable

terms, or at all. Our failure to raise capital as and when needed or on less favorable terms could have a material adverse effect on our financial condition and our ability to pursue our business strategy.

Collaboration Agreements

Baxter Healthcare SA

In September 2012, we entered into a development and license agreement with Baxter, granting Baxter an exclusive, royalty-bearing license for the research, development, commercialization and manufacture (in specified instances) of rigosertib in all therapeutic indications in Europe. Under the Baxter agreement, we are obligated to use commercially reasonable efforts to direct, coordinate and manage the development of rigosertib for MDS, in accordance with a development plan agreed upon by the parties. In addition, there is a specified mechanism set forth in the agreement to expand the scope of the collaboration for additional indications. Our agreement with Baxter is guided by a joint steering committee. If the joint steering committee is not able to make a decision by consensus, then any dispute would be resolved by specified executive officers of both parties.

Under the terms of the agreement, Baxter made an upfront payment of \$50.0 million. We are eligible to receive pre-commercial milestone payments of up to an aggregate of \$337.5 million if specified development and regulatory milestones are achieved.

We may also receive up to \$212.5 million in milestone payments for regulatory approvals of the rigosertib MDS indications specified in the arrangement with Baxter, each of which may be up to and in excess of \$100.0 million. We are also potentially eligible to receive an additional \$20.0 million pre-commercial milestone payment related to the timing of regulatory approval of rigosertib IV in higher risk MDS patients in Europe. In addition to these pre-commercial milestones, we are eligible to receive up to an aggregate of \$250.0 million in milestone payments based on Baxter's achievement of pre-specified threshold levels of annual net sales of rigosertib. We are also entitled to receive royalties at percentage rates ranging from the low-teens to the low-twenties on net sales of rigosertib by Baxter in the licensed territory. In July 2012, Baxter purchased \$50.0 million of our Series J Preferred Stock, which converted to shares of our common stock immediately prior to the consummation of the IPO. Baxter also invested \$5.0 million in our IPO in July 2013.

SymBio Pharmaceuticals Limited

In July 2011, we entered into a license agreement with SymBio, as subsequently amended, granting SymBio an exclusive, royalty-bearing license for the development and commercialization of rigosertib in Japan and Korea. Under the SymBio license agreement, SymBio is obligated to use commercially reasonable efforts to develop and obtain market approval for rigosertib inside the licensed territory, and we have similar obligations outside of the licensed territory. We have also entered into an agreement with SymBio that provides that we will supply them with development-stage product. Under the SymBio license agreement, we also agreed to supply commercial product to SymBio under specified terms that will be included in a commercial supply agreement to be negotiated prior to first commercial sale of rigosertib. We have also granted SymBio a right of first negotiation to license or obtain the rights to develop and commercialize compounds having a chemical structure similar to rigosertib in the licensed territory.

Under the terms of the SymBio license agreement, we received an upfront payment of \$7.5 million. We are eligible to receive milestone payments of up to an aggregate of \$22.0 million from SymBio upon the achievement of specified development and regulatory milestones for specified indications. Of the regulatory milestones, \$5.0 million is due upon receipt of marketing approval in the United States for rigosertib IV in higher risk MDS patients, \$3.0 million is due upon receipt of marketing approval in Japan for rigosertib IV in higher risk MDS patients, \$5.0 million is due upon receipt of marketing approval in the United States for rigosertib Oral in lower risk MDS patients, and

\$5.0 million is due upon receipt of marketing approval in Japan for rigosertib Oral in lower risk MDS patients. Furthermore, upon receipt of marketing approval in the United States and Japan for an additional specified indication of rigosertib, which we are currently not pursuing, an aggregate of \$4.0 million would be due. In addition to these pre-commercial milestones, we are eligible to receive tiered milestone payments of up to an aggregate of \$30.0 million based upon annual net sales of rigosertib by SymBio in the licensed territory.

Further, under the terms of the SymBio license agreement, SymBio is obligated to make royalty payments to us at percentage rates ranging from the mid-teens to 20% based on net sales of rigosertib by SymBio in the licensed territory.

The Leukemia and Lymphoma Society

In May 2010, we entered into a funding agreement with LLS to fund the development of rigosertib. We terminated the funding agreement effective as of March 2013. Under the LLS funding agreement, we were obligated to use the funding received exclusively for the payment or reimbursement of the costs and expenses for clinical development activities for rigosertib. Under this agreement, we retained ownership and control of all intellectual property pertaining to works of authorship, inventions, know-how, information, data and proprietary material.

Under the LLS funding agreement, as amended, we received funding of \$8.0 million from LLS through 2012. We did not receive any funding in 2013. We are required to make specified payments to LLS, including payments payable upon execution of the first out-license, first approval for marketing by a regulatory body, completion of the first commercial sale of rigosertib, and achieving specified annual net sales levels of rigosertib. The extent of these payments and our obligations will depend on whether we out-license rights to develop or commercialize rigosertib to a third party, we commercialize rigosertib on our own or we combine with or are sold to another company. In addition, we will pay to LLS a single-digit percentage royalty of our net sales of rigosertib, if any. The sum of our payments to LLS is capped at three times the total funding received from LLS, or \$24.0 million.

Financial Overview

Revenue

To date, we have derived revenue principally from activities pursuant to our collaboration arrangements with Baxter and SymBio as well as from grants and research agreements. The following table sets forth a summary of revenue recognized from our collaboration agreements and research agreements for the years ended December 31, 2013, 2012 and 2011:

	Year Ended December 31,		
	2013	2012	2011
Baxter license and collaboration revenue	\$ 4,176,000	\$ 45,490,000	\$ —
SymBio license and collaboration revenue	577,000	503,000	227,000
Research funding	—	197,000	1,260,000
	<u>\$ 4,753,000</u>	<u>\$ 46,190,000</u>	<u>\$ 1,487,000</u>

We have not generated any revenue from commercial product sales. In the future, if any of our product candidates currently under development are approved for commercial sale in the United States and Canada, we may generate revenue from product sales, or alternatively, we may choose to select a collaborator to commercialize our product candidates in these markets.

The Baxter collaboration agreement is considered to be a multiple-element arrangement for accounting purposes. We determined that there are two deliverables under the Baxter agreement;

specifically, the license to rigosertib for Europe and the related research and development services that we are obligated to provide. We concluded that \$42.4 million of the fixed and determinable \$50.0 million upfront payment was associated with the license and \$7.6 million was associated with the research and development services. We recognized the entire \$42.4 million associated with the upfront license as revenue during the third quarter of 2012 upon the execution of the Baxter agreement, and we are recognizing the research and development services revenue of \$7.6 million on the proportional performance method over the period of commitment and development, which we estimate to be through March 31, 2014, the period of our non-contingent obligations to perform research and development services sufficient to advance rigosertib. For the years ended December 31, 2013, 2012 and 2011, we recognized \$4.2 million, \$3.1 million and \$0.0 million, respectively, of research and development services revenue under the Baxter agreement.

The SymBio collaboration agreement is also considered to be a multiple-element arrangement for accounting purposes. We determined that there were three deliverables under the SymBio collaboration agreement; specifically, the license to rigosertib for Japan and Korea, our obligation to perform research and development services necessary for SymBio to seek approval in its territory and our obligation to participate on a joint steering committee. We concluded that these deliverables should be accounted for as a single unit of accounting. We determined that the \$7.5 million upfront payment received in 2011 should be deferred and recognized as revenue on a straight-line basis through December 2027, reflecting our estimate of when we will complete our obligations under the agreement. For the years ended December 31, 2013, 2012 and 2011, we recognized revenues of \$455,000, \$455,000 and \$227,000, respectively, under the SymBio collaboration agreement. In addition, we recognized revenues of \$122,000, \$48,000 and \$0.0 for the years ended December 31, 2013, 2012 and 2011, respectively, related to the supply agreement with SymBio.

Pursuant to our funding agreement with LLS, during the year ended December 31, 2012, we paid \$1.0 million to LLS, which we recorded as research and development expenses. This payment reduced the maximum milestone and royalty payment obligation under this agreement to \$23.0 million at December 31, 2013.

In addition, some of our obligations under the LLS funding agreement will remain in effect until the completion of specified milestones and payments to LLS. Assuming the successful outcome of the development activities covered by the LLS funding agreement and our receipt of necessary regulatory approvals, we will be required to take commercially reasonable steps through March 2018 to advance the development of rigosertib in clinical trials and to bring rigosertib to practical application for MDS in a major market country, provided that we believe the product is safe and effective. We believe that we can satisfy our obligation by out-licensing rigosertib to, or partnering rigosertib with, a third party. We are required to report to LLS on our efforts and results with respect to continuing development of rigosertib. Our failure to perform these diligence obligations, even if we successfully achieve the specified development milestones, would require us to pay back to LLS the total amount of the funding we received from them, unless an exception applies. If LLS were to claim that such failure occurred and we disagreed with such claim, the dispute would be settled through binding arbitration.

As a result of the potential obligation to pay back to LLS the total amount of funding received under this arrangement, the \$8.0 million of milestone payments we received through December 31, 2013 has been recorded as deferred revenue.

Preclinical Collaboration

In December 2012, we formed GBO, LLC, or GBO, an entity jointly-owned by both us and GVK Biosciences Private Limited, or GVK, to collaborate on the development of two of our preclinical programs. GVK made an initial capital contribution of \$500,000 in exchange for a 10% interest in GBO and we contributed a sub-license to the intellectual property related to the two programs in

exchange for a 90% interest. GVK will be required to make additional capital contributions over time, subject to specified conditions, and its interest in GBO will increase to as much as 50%. At specified times, we will be entitled to buy back from GVK the rights to either of these two programs.

GBO is consolidated in our financial statements for the year ended December 31, 2013, which means that we included its assets and liabilities in our balance sheets and its expenses in our statements of operations, less those amounts comprising the non-controlling interest. The consolidation of GBO did not have a material effect on our consolidated financial position or results of operations.

Operating Expenses

The following table summarizes our operating expenses for the years ended December 31, 2013, 2012 and 2011:

	<u>2013</u>	<u>2012</u>	<u>2011</u>
General and administrative	\$ 16,793,000	\$ 15,707,000	\$ 6,436,000
Research and development	50,182,000	52,762,000	22,624,000
Total operating expenses	<u>\$ 66,975,000</u>	<u>\$ 68,469,000</u>	<u>\$ 29,060,000</u>

General and Administrative Expenses

General and administrative expenses consist principally of salaries and related costs for executive and other administrative personnel, including stock-based compensation and travel expenses. Other general and administrative expenses include facility-related costs, communication expenses and professional fees for legal, patent review, consulting and accounting services.

We anticipate that our general and administrative expenses will increase in the future with the continued research and development and potential commercialization of our product candidates and as we operate as a public company. These increases will likely include increased costs for insurance, costs related to the hiring of additional personnel and payments to outside consultants, investor relations, lawyers and accountants, among other expenses. Additionally, if and when we believe a regulatory approval of a product candidate appears likely, we anticipate an increase in payroll and expense as a result of our preparation for commercial operations, especially as it relates to the sales and marketing of our product candidates.

Research and Development Expenses

Our research and development expenses consist primarily of costs incurred for the development of our product candidates, which include:

- employee-related expenses, including salaries, benefits, travel and stock-based compensation expense;
- expenses incurred under agreements with CROs and investigative sites that conduct our clinical trials and preclinical studies;
- the cost of acquiring, developing and manufacturing clinical trial materials;
- facilities, depreciation and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance and other supplies; and
- costs associated with preclinical activities and regulatory operations.

Research and development costs are expensed as incurred. License fees and milestone payments we make related to in-licensed products and technology are expensed if it is determined that they have

no alternative future use. We record costs for some development activities, such as clinical trials, based on an evaluation of the progress to completion of specific tasks using data such as patient enrollment, clinical site activations or information provided to us by our vendors.

Research and development activities are central to our business model. Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. We plan to increase our research and development expenses for the foreseeable future.

To date, our research and development expenses have related primarily to the development of rigosertib. We do not currently utilize a formal time allocation system to capture expenses on a project-by-project basis because we are organized and record expense by functional department and our employees may allocate time to more than one development project. Accordingly, we do not allocate expenses to individual projects or product candidates, although we do allocate some portion of our research and development expenses by functional area and by compound.

The following table summarizes our research and development expenses by functional area for the years ended December 31, 2013, 2012 and 2011:

	Twelve Months Ended December 31,		
	2013	2012	2011
Pre-clinical & clinical development	\$ 28,693,000	\$ 20,957,000	\$ 11,593,000
Milestones & royalties	—	13,500,000	1,875,000
Personnel related	9,059,000	4,876,000	4,020,000
Manufacturing, formulation & development	4,967,000	3,362,000	3,184,000
Stock-based compensation	3,170,000	6,645,000	3,000
Consulting fees	4,293,000	3,422,000	1,949,000
	<u>\$ 50,182,000</u>	<u>\$ 52,762,000</u>	<u>\$ 22,624,000</u>

It is difficult to determine with certainty the duration and completion costs of our current or future preclinical programs and clinical trials of our product candidates, or if, when or to what extent we will generate revenues from the commercialization and sale of any of our product candidates that obtain regulatory approval. We may never succeed in achieving regulatory approval for any of our product candidates. The duration, costs and timing of clinical trials and development of our product candidates will depend on a variety of factors, including the uncertainties of future clinical and preclinical studies, uncertainties in clinical trial enrollment rate and significant and changing government regulation. In addition, the probability of success for each product candidate will depend on numerous factors, including competition, manufacturing capability and commercial viability. We will determine which programs to pursue and how much to fund each program in response to the scientific and clinical success of each product candidate, as well as an assessment of each product candidate's commercial potential.

Change in Fair Value of Warrant Liability

We issued warrants for the purchase of our Series G convertible preferred stock that we believed were financial instruments that might require a transfer of assets because of the redemption features of the underlying preferred stock. Therefore, we classified these warrants as liabilities that we re-measured to fair value at each balance sheet date and we recorded the changes in the fair value of the warrant liability as either income or expense. Upon consummation of our IPO, the underlying preferred stock was converted to common stock, and the fair value of the warrant liability at that time was reclassified to additional paid-in capital.

Interest Expense and Other Income, Net

Other income, net consists principally of interest income earned on cash and cash equivalent balances and income earned on our sale of New Jersey state net operating losses in 2012.

Interest expense for the years ended December 31, 2013, 2012 and 2011 consisted of cash paid and non-cash interest expense related to our prior loan from a stockholder and convertible promissory notes payable, as well as a charge of \$8.2 million for the unamortized contingent beneficial conversion feature upon conversion of those promissory notes into shares of Series I convertible preferred stock in 2012.

Accretion of Preferred Stock

We accounted for the redemption of premium and issuance costs on our preferred stock using the interest method, accreting such amounts to preferred stock from the date of issuance to the earliest date of redemption. Immediately prior to the consummation of our IPO, all series of our preferred stock converted into common stock.

Critical Accounting Policies and Significant Judgments and Estimates

This management's discussion and analysis of our financial condition and results of operations is based on our consolidated financial statements, which have been prepared in accordance with GAAP. The preparation of these financial statements requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, revenues and expenses and the disclosure of contingent assets and liabilities in our consolidated financial statements. On an ongoing basis, we evaluate our estimates and judgments, including those related to accrued expenses, revenue recognition, deferred revenue and stock-based compensation. We base our estimates on historical experience, known trends and events and various other factors that we believe to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are described in the notes to our consolidated financial statements appearing elsewhere in this prospectus, we believe the following accounting policies to be most critical to the judgments and estimates used in the preparation of our consolidated financial statements.

Revenue Recognition

We generate revenue primarily through collaborative research and license agreements. The terms of these agreements contain multiple deliverables, which may include licenses, research and development activities, participation in joint steering committees and product supply. The terms of these agreements may include nonrefundable upfront license fees, payments for research and development activities, payments based upon the achievement of specified milestones, royalty payments based on product sales derived from the collaboration, and payments for supplying product. In all instances, we recognize revenue only when the price is fixed or determinable, persuasive evidence of an arrangement exists, delivery has occurred or the services have been rendered, collectability of the resulting receivable is reasonably assured and we have fulfilled our performance obligations under the contract.

Effective January 1, 2011, we adopted the Financial Accounting Standards Board, or FASB, Accounting Standards Update, or ASU, No. 2009-13, *Multiple-Deliverable Revenue Arrangements*, or ASU 2009-13. This guidance, which applies to multiple-element arrangements entered into or materially modified on or after January 1, 2011, amends the criteria for separating and allocating consideration in

a multiple-element arrangement by modifying the fair value requirements for revenue recognition and eliminating the use of the residual value method. The selling prices of deliverables under an arrangement may be derived using third-party evidence, or TPE, or a best estimate of selling price, or BESP, if vendor-specific objective evidence of fair value, or VSOE, is not available. The objective of BESP is to determine the price at which we would transact a sale if the element within the license agreement was sold on a standalone basis. Establishing BESP involves management's judgment and takes into account multiple factors, including market conditions and company-specific factors, such as those factors contemplated in negotiating the agreements as well as internally developed models that include assumptions related to market opportunity, discounted cash flows, estimated development costs, probability of success, and the time needed to commercialize a product candidate pursuant to the license. In validating the BESP, management considers whether changes in key assumptions used to determine the BESP will have a significant effect on the allocation of the arrangement consideration between the multiple deliverables. We may use third-party valuation specialists to assist us in determining BESP. Deliverables under the arrangement are separate units of accounting if (i) the delivered item has value to the customer on a standalone basis and (ii) if the arrangement includes a general right of return relative to the delivered item, delivery or performance of the undelivered item is considered probable and substantially within our control. The arrangement consideration that is fixed or determinable at the inception of the arrangement is allocated to the separate units of accounting based on their relative selling prices. The appropriate revenue recognition model is applied to each element and revenue is accordingly recognized as each element is delivered. Management exercises significant judgment in determining whether a deliverable is a separate unit of accounting.

In determining the separate units of accounting, we evaluate whether the license has standalone value to the collaborator based on consideration of the relevant facts and circumstances for each arrangement. Factors considered in this determination include the research and development capabilities of the collaborator and the availability of relevant research expertise in the marketplace. In addition, we consider whether or not (i) the collaborator can use the license for its intended purpose without the receipt of the remaining deliverables, (ii) the value of the license is dependent on the undelivered items and (iii) the collaborator or other vendors can provide the undelivered items.

Under a collaborative research and license agreement, a steering committee is typically responsible for overseeing the general working relationships, determining the protocols to be followed in the research and development performed, and evaluating the results from the continued development of the product. We evaluate whether our participation in joint steering committees is a substantive obligation or whether the services are considered inconsequential or perfunctory. The factors we consider in determining if our participation in a joint steering committee is a substantive obligation include: (i) which party negotiated or requested the steering committee, (ii) how frequently the steering committee meets, (iii) whether or not there are any penalties or other recourse if we do not attend the steering committee meetings, (iv) which party has decision making authority on the steering committee and (v) whether or not the collaborator has the requisite experience and expertise associated with the research and development of the licensed intellectual property.

For all periods presented, whenever we determine that an element is delivered over a period of time, we recognize revenue using either a proportional performance model or a straight-line model over the period of performance, which is typically the research and development term. We typically use progress achieved under our various CRO contracts as the measure of performance. At each reporting period, we reassess our cumulative measure of performance and make appropriate adjustments, if necessary. We recognize revenue using the proportional performance model whenever we can make reasonably reliable estimates of the level of effort required to complete our performance obligations under an arrangement. We recognize revenue under the proportional performance model at each reporting period by multiplying the total expected payments under the contract, excluding royalties and payments contingent upon achievement of milestones, by the ratio of the level of effort incurred to

date to the estimated total level of effort required to complete the performance obligations under the arrangement. Revenue is limited to the lesser of the cumulative amount of payments received or the cumulative amount of revenue earned, as determined using the proportional performance model as of each reporting period. Alternatively, if we cannot make reasonably reliable estimates of the level of effort required to complete our performance obligations under an arrangement, then we recognize revenue under the arrangement on a straight-line basis over the period expected to complete our performance obligations.

Incentive milestone payments may be triggered either by the results of our research efforts or by events external to us, such as regulatory approval to market a product. We recognize consideration that is contingent upon achievement of a milestone in its entirety as revenue in the period in which the milestone is achieved, but only if the consideration earned from the achievement of a milestone meets all the criteria for the milestone to be considered substantive at the inception of the arrangement. For a milestone to be considered substantive, the consideration earned by achieving the milestone must be commensurate with either our performance to achieve the milestone or the enhancement of the value of the item delivered as a result of a specific outcome resulting from our performance to achieve the milestone, relate solely to our past performance and be reasonable relative to all deliverables and payment terms in the collaboration agreement.

For events for which the occurrences are contingent solely upon the passage of time or are the result of performance by a third party, we will recognize the contingent payments as revenue when payments are earned, the amounts are fixed and determinable and collectability is reasonably assured.

We will recognize royalty revenue, if any, as earned in accordance with the contract terms when third-party sales can be reliably measured and collectability is reasonably assured.

We recognized revenue of \$4.2 million, \$45.5 million and \$0.0 million during the years ended December 31, 2013, 2012 and 2011, respectively, under our license and collaboration agreement with Baxter. We recognized revenue of \$577,000, \$503,000 and \$227,000 during the years ended December 31, 2013, 2012 and 2011, respectively, under our license and collaboration agreement with SymBio. The remaining revenue recognized during the years ended December 31, 2013, 2012 and 2011 of \$0, \$197,000 and \$1,260,000, respectively, pertained to research and development services provided under research grants. The Baxter and SymBio agreements are the only agreements that are being accounted for under ASU 2009-13.

Research and Development Expenses

Research and development costs are charged to expense as incurred and include, but are not limited to, license fees related to the acquisition of in-licensed products, employee-related expenses, including salaries, benefits and travel, expenses incurred under agreements with CROs and investigative sites that conduct clinical trials and preclinical studies, the cost of acquiring, developing and manufacturing clinical trial materials, facilities, depreciation and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance and other supplies and costs associated with preclinical activities and regulatory operations.

We record costs for certain development activities, such as clinical trials, based on our evaluation of the progress to completion of specific tasks using data such as patient enrollment, clinical site activations, or information provided to us by our vendors on their actual costs incurred. Payments for these activities are based on the terms of the individual arrangements, which may differ from the pattern of costs incurred, and are reflected in the consolidated financial statements as prepaid or accrued research and development expense, as the case may be.

Income Taxes

We recorded deferred tax assets of \$93.3 million as of December 31, 2013, which have been fully offset by a valuation allowance due to uncertainties surrounding our ability to realize these tax benefits. The deferred tax assets are primarily composed of federal and state tax net operating loss, or NOL, carry forwards and research and development tax credit carry forwards. As of December 31, 2013, we had federal NOL carry forwards of \$119.2 million, state NOL carry forwards of \$99.3 million and research and development tax credit carry forwards of \$35.1 million available to reduce future taxable income, if any. These federal NOL carry forwards will begin to expire at various dates starting in 2022. The state NOL carry forwards will begin to expire at various dates starting in 2016. In general, if we experience a greater than 50 percentage point aggregate change in ownership of specified significant stockholders over a three-year period, utilization of our pre-change NOL carry forwards will be subject to an annual limitation under Section 382 of the U.S. Internal Revenue Code of 1986, as amended, or the Code, and similar state laws. Such limitations may result in expiration of a portion of the NOL carry forwards before utilization and may be substantial. We have determined that we have experienced ownership changes in the past and approximately \$24.0 million of our NOL carry forwards are subject to an annual limitation under Section 382 of the Code. If we experienced a Section 382 ownership change in connection with the offering or as a result of future changes in our stock ownership, some of which changes are outside our control, the tax benefits related to the NOL carry forwards may be further limited or lost.

Stock-Based Compensation

Prior to April 2013, our stock option awards were accounted for as liability awards as we, through our chairman of the board of directors, who is also a significant stockholder, had established a pattern of settling these awards for cash. Accordingly, we measured stock-based compensation expense at the end of each reporting period based on the intrinsic value of all outstanding vested stock options on each reporting date and recognized expense based on any increases in their intrinsic value since the last measurement date to the extent the stock options vested. The intrinsic value represented the difference between the current fair value of our common stock and the contractual exercise prices of the awards.

On April 23, 2013, we distributed a notification letter to all holders of stock options under our 2007 Equity Compensation Plan advising them that cash settlement transactions would no longer occur, unless, at the time of a cash settlement transaction, the option holder has held the common stock issued upon exercise of options for a period of greater than six months prior to such cash settlement transaction and that any such settlement would be at the fair value of the common stock on the date of such sale. Following this notification, we reclassified options outstanding under our 2007 Equity Compensation Plan from liabilities to stockholders' deficit within our consolidated balance sheets. Upon issuing the notification, a modification to the liability awards occurred and the awards are now accounted for as equity awards from the date of modification with compensation expense fixed at fair value at the modification date. As a result, we reclassified the amount of stock-based compensation liability at the modification date to additional paid-in capital. The modification date fair value is recognized over the remaining service period, generally the vesting period, on a straight-line basis. The fair value of the modified awards was estimated on the modification date using the intrinsic value model. The grant date fair value of awards granted after the modification is estimated using the Black-Scholes valuation model, net of estimated forfeitures. Awards granted to non-employees will also be valued using the Black-Scholes valuation model and will be subject to periodic adjustment until the underlying equity instruments vest.

Stock-based compensation expense totaled \$8.0 million, \$13.8 million and \$6,000 for the years ended December 31, 2013, 2012 and 2011, respectively. We record stock-based compensation expense as a component of research and development expenses or general and administrative expenses,

depending on the function performed by the optionee. For the years ended December 31, 2013, 2012 and 2011, we allocated stock-based compensation as follows:

	2013	2012	2011
General and administrative	\$ 4,845,000	\$ 7,199,000	\$ 3,000
Research and development	3,170,000	6,645,000	3,000
Total	<u>\$ 8,015,000</u>	<u>\$ 13,844,000</u>	<u>\$ 6,000</u>

Fair Value Estimates

Since April 23, 2013, we estimate the fair value of share-based awards to employees and directors using the Black-Scholes option pricing model. The Black-Scholes model requires the input of highly complex and subjective assumptions, including (a) the expected stock price volatility, (b) the calculation of the expected term of the award, (c) the risk free interest rate and (d) expected dividends. Due to our limited operating history and a lack of company specific historical and implied volatility data, we based our estimate of expected volatility on the historical volatility of a group of similar companies that are publicly traded. When selecting these public companies on which we have based its expected stock price volatility, we selected companies with comparable characteristics to us, including enterprise value, risk profiles, position within the industry, and with historical share price information sufficient to meet the expected life of the stock-based awards. The historical volatility data was computed using the daily closing prices for the selected companies' shares during the equivalent period of the calculated expected term of the stock-based awards. Due to our lack of sufficient historical data, we will continue to apply this process until a sufficient amount of historical information regarding the volatility of our own stock price becomes available. We estimate the expected life of our employee stock options using the "simplified" method, whereby, the expected life equals the arithmetic average of the vesting term and the original contractual term of the option. The risk-free interest rates for periods within the expected life of the option are based on the U.S. Treasury yield curve in effect during the period the options were granted. We have never paid, and do not expect to pay dividends in the foreseeable future.

Prior to April 23, 2013, we were required to estimate the fair value of the common stock underlying our stock-based awards when performing the fair value calculations using the intrinsic value method at each reporting date. In the absence of a public trading market for our common stock, on each grant date, we developed an estimate of the fair value of our common stock by engaging an independent third-party valuation firm to assist our board of directors in determining the fair value of the common stock underlying our stock-based awards. We determined the fair value of our common stock using methodologies, approaches and assumptions consistent with the American Institute of Certified Public Accountants, or AICPA, Audit and Accounting Practice Aid Series: *Valuation of Privately Held Company Equity Securities Issued as Compensation*, or the AICPA Practice Guide. All options to purchase shares of our common stock were granted with an exercise price per share no less than the fair value per share of our common stock underlying those options on the date of grant, based on the information known to us on the date of grant. Accordingly, under the liability method of accounting, we did not record any stock-based compensation expense on the grant dates of our options. However, under the liability method, the liability for all outstanding vested stock-based awards was adjusted through our statement of operations, based on the current estimated fair value of our common stock at each reporting date.

Clinical Trial Expense

As part of the process of preparing our consolidated financial statements, we are required to estimate our accrued expenses. Our clinical trial accrual process is designed to account for expenses

resulting from our obligations under contracts with vendors, consultants and CROs and clinical site agreements in connection with conducting clinical trials. The financial terms of these contracts are subject to negotiations, which vary from contract to contract and may result in payment flows that do not match the periods over which materials or services are provided to us under such contracts. Our objective is to reflect the appropriate clinical trial expenses in our consolidated financial statements by matching the appropriate expenses with the period in which services are provided and efforts are expended. We account for these expenses according to the progress of the trial as measured by patient progression and the timing of various aspects of the trial. We determine accrual estimates through financial models that take into account discussion with applicable personnel and outside service providers as to the progress or state of completion of trials, or the services completed. During the course of a clinical trial, we adjust our clinical expense recognition if actual results differ from our estimates. We make estimates of our accrued expenses as of each balance sheet date in our consolidated financial statements based on the facts and circumstances known to us at that time. Our clinical trial accrual and prepaid assets are dependent, in part, upon the receipt of timely and accurate reporting from CROs and other third-party vendors. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and may result in us reporting amounts that are too high or too low for any particular period.

JOBS Act

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

Results of Operations

Comparison of Years Ended December 31, 2013 and 2012

	<u>Year ended December 31,</u>		<u>Change</u>
	<u>2013</u>	<u>2012</u>	
Revenue	\$ 4,753,000	\$ 46,190,000	\$ (41,437,000)
Operating expenses:			
General and administrative	16,793,000	15,707,000	(1,086,000)
Research and development	50,182,000	52,762,000	2,580,000
Total operating expenses	<u>66,975,000</u>	<u>68,469,000</u>	<u>1,494,000</u>
Loss from operations	(62,222,000)	(22,279,000)	(39,943,000)
Change in fair value of warrant liability	42,000	367,000	(325,000)
Interest expense	(4,000)	(8,608,000)	8,604,000
Other income (expense), net	63,000	608,000	(545,000)
Net loss before income taxes	(62,121,000)	(29,912,000)	(32,209,000)
Income taxes	435,000	—	(435,000)
Net loss	<u>\$ (62,556,000)</u>	<u>\$ (29,912,000)</u>	<u>\$ (32,644,000)</u>

Revenues

Revenues decreased by \$41.4 million in 2013 when compared to 2012 primarily as a result of entering into the Baxter agreement in 2012. Of this decrease, \$42.4 million was attributable to the revenue recognized upon signing of the Baxter agreement in 2012, offset by the revenue recognized related to development services under the Baxter agreement, which increased by \$1.1 million to \$4.2 million in 2013 from \$3.1 million in 2012. Research funding decreased by \$0.2 million in 2013 compared to 2012.

General and administrative expenses

General and administrative expenses increased by \$1.1 million, or 6.9%, to \$16.8 million for the year ended December 31, 2013 compared to \$15.7 million for the year ended December 31, 2012. The increase was attributable to an increase of \$1.6 million as a result of general and administrative headcount growing from 10 at the end of 2012 to 15 at the end of 2013. Insurance, meetings, and facilities-related expenses increased \$1.6 million as total Company-wide headcount grew from 46 at December 31, 2012 to 64 at December 31, 2013 and because of increased operating costs as a result of becoming a public company. Professional fees remained relatively flat as fees related to negotiating the Baxter agreement in 2012 were replaced by fees related to being a public company in 2013. These increases in general and administrative expenses were offset by a decrease of \$2.4 million in stock-based compensation which was primarily the result of a change in accounting method during 2013.

Research and development expenses

Research and development expenses decreased by \$2.6 million, or 4.9%, to \$50.2 million for the year ended December 31, 2013 compared to \$52.8 million for the year ended December 31, 2012. This decrease was driven primarily by a \$12.5 million milestone paid to Temple University as a result of entering into the Baxter agreement and a \$1.0 million payment to LLS in 2012, as well as a decrease in stock-based compensation of \$3.5 million in 2013 compared to 2012 due primarily to a change in accounting method during 2013. These decreases were partially offset by an increase in pre-clinical and clinical trial expenses of \$6.3 million; an increase of \$4.2 million related to a change in research and development headcount to 46 at December 31, 2013 from 36 at December 31, 2012; an increase of \$3.0 million in manufacturing, formulation, and development costs; and an increase of \$0.9 million related to consulting services in connection with clinical development.

Change in fair value of warrant liability

The fair value of the warrant liability decreased by \$42,000 during the year ended December 31, 2013 compared to a decrease of \$0.4 million during the year ended December 31, 2012, which in both cases resulted in a commensurate increase in other income. The decrease in the fair value of the warrant liability in 2013 was primarily due to the revaluation of the warrants outstanding. The decrease in the fair value of the warrant liability in 2012 was primarily due a decrease of \$1.0 million related to the expiration of Series G convertible preferred stock warrants in 2012, offset by an increase of \$0.6 million related to the revaluation of the warrants outstanding.

Interest expense

Interest expense decreased by \$8.6 million during the year ended December 31, 2013 compared to the year ended December 31, 2012. In July 2012, the holders of our convertible notes elected to convert their outstanding principal and accrued interest into shares of Series I convertible preferred stock. At the time of the conversion, there was \$8.2 million in unamortized contingent beneficial conversion features that we immediately expensed.

Other income, net

Other income, net, decreased by \$0.5 million during the year ended December 31, 2013 compared to the year ended December 31, 2012. This decrease was driven by a \$0.5 million gain recognized on our sale of New Jersey state NOL carry forwards in 2012.

Comparison of Years Ended December 31, 2012 and 2011

	<u>Year ended December 31,</u>		<u>Change</u>
	<u>2012</u>	<u>2011</u>	
Revenue	\$ 46,190,000	\$ 1,487,000	\$ 44,703,000
Operating expenses:			
General and administrative	15,707,000	6,436,000	(9,271,000)
Research and development	52,762,000	22,624,000	(30,138,000)
Total operating expenses	<u>68,469,000</u>	<u>29,060,000</u>	<u>(39,409,000)</u>
Loss from operations	(22,279,000)	(27,573,000)	5,294,000
Change in fair value of warrant liability	367,000	1,287,000	(920,000)
Interest expense	(8,608,000)	(19,000)	(8,589,000)
Other income (expense), net	608,000	11,000	597,000
Net loss before income taxes	(29,912,000)	(26,294,000)	(3,618,000)
Income taxes	—	—	—
Net loss	<u>\$ (29,912,000)</u>	<u>\$ (26,294,000)</u>	<u>\$ (3,618,000)</u>

Revenues

Revenues increased by \$44.7 million in 2012 when compared to 2011 primarily as a result of entering into the Baxter agreement in 2012. Of this increase, \$42.4 million related to the value ascribed to the license, and was therefore recognized immediately upon signing of the Baxter agreement, and \$3.1 million related to the portion of development services revenue recognized in 2012 under the Baxter agreement. Those increases attributable to the Baxter agreement were partially offset by a \$1.1 million reduction in research funding.

General and administrative expenses

General and administrative expenses increased by \$9.3 million, or 144.0%, to \$15.7 million for the year ended December 31, 2012 from \$6.4 million for the year ended December 31, 2011. The increase was primarily attributable to an increase of \$7.2 million related to stock-based compensation due to the increase in the fair value of our common stock during the year, an increase of \$1.7 million in professional fees related to the negotiation of the Baxter agreement in 2012, and an increase of \$0.4 million as a result of the increase in general and administrative headcount from six at the end of 2011 to nine at the end of 2012.

Research and development expenses

Research and development expenses increased by \$30.1 million, or 133.0%, to \$52.8 million for the year ended December 31, 2012 from \$22.6 million for the year ended December 31, 2011. This increase was driven by a \$12.5 million milestone due to Temple, in 2012 as a result of entering into the Baxter agreement in 2012 compared to a \$1.9 million payment to Temple in 2011 as a result of entering into the SymBio agreement. The change was also due to an increase in clinical trial expenses of \$8.4 million for rigosertib, \$6.6 million in additional stock-based compensation due to the increase in the fair value of our common stock during the year, an increase of \$1.4 million related to consulting services in

connection with the rigosertib clinical trials, an increase of \$1.5 million in nonclinical trial-related costs for rigosertib and an increase of \$0.9 million related to a change in research and development headcount to 36 at the end of 2012 from 25 at the end of 2011 as a result of our expanded research and development activities.

Change in fair value of warrant liability

The fair value of the warrant liability decreased by \$0.4 million during the year ended December 31, 2012 compared to a decrease of \$1.3 million during the year ended December 31, 2011, which in both cases resulted in a commensurate increase in other income. The decrease in the fair value of the warrant liability in 2012 was primarily due to the expiration of Series G convertible preferred stock warrants in 2012, which accounted for a decrease of \$1.0 million in the value of the liability, which was partially offset by an increase of \$0.6 million in the value of the liability related to the revaluation of the warrants outstanding. The decrease in the fair value of the warrant liability in 2011 was primarily due to the revaluation of the warrants outstanding.

Interest expense

Interest expense increased by \$8.6 million during the year ended December 31, 2012 compared to the year ended December 31, 2011. In July 2012, the holders of our convertible notes elected to convert their outstanding principal and accrued interest into shares of Series I convertible preferred stock. At the time of the conversion, there was \$8.2 million in unamortized contingent beneficial conversion features that we immediately expensed.

Other income, net

Other income, net, increased by \$0.6 million during the year ended December 31, 2012 compared to the year ended December 31, 2011. This increase was driven by a \$0.5 million gain recognized on our sale of New Jersey state NOL carry forwards.

Liquidity and Capital Resources

Since our inception, we have incurred net losses and generally negative cash flows from our operations. We incurred net losses of \$62.6 million, \$29.9 million, and \$26.3 million for the years ended December 31, 2013, 2012 and 2011, respectively. Since inception our accumulated deficit is \$231 million.

Cash Flows

The following table summarizes our cash flows for the years ended December 31, 2013, 2012 and 2011:

	Year Ended December 31,		
	2013	2012	2011
Net cash (used in) provided by:			
Operating activities	\$ (61,384,000)	\$ 1,633,000	\$ (14,171,000)
Investing activities	(40,599,000)	(279,000)	(241,000)
Financing activities	80,464,000	77,460,000	9,785,000
Effect of foreign currency translation on cash	1,000	—	—
Net (decrease) increase in cash and cash equivalents	\$ (21,518,000)	\$ 78,814,000	\$ (4,627,000)

Net cash (used in) provided by operating activities

Net cash used in operating activities was \$61.4 million for the year ended December 31, 2013 and consisted primarily of a net loss of \$62.6 million and a decrease in deferred revenue of \$4.6 million related to the recognition of deferred revenue under the Baxter and Symbio collaboration agreements. These were offset by \$8.4 million of noncash increases primarily related to stock compensation expense of \$8.0 million and depreciation of \$0.4 million. The cash used in operating activities was also impacted by the changes in operating assets and liabilities including an increase in prepaid expenses and other current assets of \$2.7 million which was the result of the timing of expense recognition and payments to our contract research and manufacturing organizations, and an increase of \$0.1 million in accounts payable and accrued expenses, which was primarily due to the timing of our payments of clinical trial costs related to the ongoing trials and development of our product candidates.

Net cash provided by operating activities was \$1.6 million for the year ended December 31, 2012 and consisted primarily of noncash increases of \$22.0 million and a \$9.6 million increase related to the change in operating assets and liabilities that were offset by a net loss of \$29.9 million. The noncash increases were primarily attributable to increases in stock-based compensation and recognition of debt discounts and beneficial conversion features of \$8.2 million upon conversion of our convertible promissory notes into preferred stock. The significant items in the change in operating assets and liabilities included an increase in prepaid expenses and other current assets of \$1.1 million, offset by increases in accrued expenses of \$2.6 million and an increase in deferred revenues of \$8.2 million. The increase in prepaid expenses and other current assets was primarily due to the prepayment of upfront costs for our Phase 3 clinical trials and continued development activities. The increase in accrued expenses was primarily due to the costs for our Phase 3 clinical trial activities. The increase in deferred revenues was primarily due to the receipt of payments from Baxter and LLS of \$7.6 million and \$4.1 million, respectively, pursuant to the terms of our agreements with such parties. These increases were partially offset by the recognition of the unamortized portions of upfront payments under our collaboration agreements with Baxter and Symbio for \$3.1 million and \$0.5 million, respectively.

Net cash used in investing activities

Net cash used in investing activities for the years ended December 31, 2013 and 2012 was \$40.6 million and \$0.3 million, respectively. Cash used in investing activities during 2013 consisted of the purchases of marketable securities of \$40.0 million and purchases of fixed assets of \$0.6 million. Cash used in investing activities during 2012 consisted primarily of purchases of fixed assets.

Net cash provided by financing activities

Net cash provided by financing activities was \$80.5 million for the year ended December 31, 2013, which was primarily due to \$79.8 million in net proceeds from the IPO, the \$0.5 million investment by our collaboration partner, GVK, and \$0.2 million in proceeds upon the exercise of stock options.

Net cash provided by financing activities was \$77.5 million for the year ended December 31, 2012, which was primarily due to \$47.8 million in proceeds from the issuance of Series J convertible preferred stock in connection with the Baxter equity investment, \$25.8 million in proceeds upon the issuance of convertible debt that was subsequently converted into shares of Series I convertible preferred stock, \$2.2 million in proceeds upon the exercise of warrants in exchange for shares of Series G convertible preferred stock, \$0.4 million in proceeds from collection of a subscription receivable for our issuance of Series H convertible preferred stock and \$1.3 million in proceeds upon the exercise of stock options.

Operating and Capital Expenditure Requirements

We have not achieved profitability since our inception and we expect to continue to incur net losses for the foreseeable future. We expect our cash expenditures to increase in the near term as we fund our Phase 2 and Phase 3 clinical trials of rigosertib, as well as our clinical trials of our other earlier-stage product candidates and continuing preclinical activities.

On July 30, 2013, we completed our IPO. We received net proceeds of \$79.8 million from the sale, net of underwriting discounts and commissions and other estimated offering expenses.

As a publicly traded company, we have incurred and will continue to incur significant legal, accounting and other expenses that we were not required to incur as a private company. In addition, the Sarbanes-Oxley Act of 2002, as well as rules adopted by the SEC and the NASDAQ Stock Market, require public companies to implement specified corporate governance practices that are currently inapplicable to us as a private company. We expect these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. We estimate that we will incur approximately \$2.0 million to \$3.0 million of incremental costs per year associated with being a publicly traded company, although it is possible that our actual incremental costs will be higher than we currently estimate.

We believe that our existing capital resources, together with the net proceeds from our IPO, will be sufficient to fund our operations for at least the next 12 months. However, we anticipate that we will need to raise substantial additional financing in the future to fund our operations. In order to meet these additional cash requirements, we may seek to sell additional equity or convertible debt securities that may result in dilution to our stockholders. If we raise additional funds through the issuance of convertible debt securities, these securities could have rights senior to those of our common stock and could contain covenants that restrict our operations. There can be no assurance that we will be able to obtain additional equity or debt financing on terms acceptable to us, if at all. Further, the achievement of milestones and receipt from Baxter and SymBio of milestone payments and royalties, even if rigosertib is approved for commercial use in Baxter's and SymBio's licensed territories, are not assured. Our failure to obtain sufficient funds on acceptable terms when needed could have a negative impact on our business, results of operations, and financial condition. Our future capital requirements will depend on many factors, including:

- the results of our Phase 2 and Phase 3 clinical trials;
- whether Baxter and SymBio continue to pursue or terminate our collaboration arrangements for the development and commercialization of rigosertib in their licensed territories;
- the amount and timing of any milestone payments or royalties we may receive pursuant to our collaboration arrangements;
- the number and characteristics of any other product candidates we develop or may acquire;
- the scope, progress, results and costs of researching and developing our product candidates or any future product candidates, and conducting preclinical and clinical trials;
- the timing of, and the costs involved in, obtaining regulatory approvals for our product candidates or any future product candidates;
- the cost of commercialization activities if any future product candidates are approved for sale, including marketing, sales and distribution costs;
- the cost of manufacturing rigosertib and our other product candidates and any products that may achieve regulatory approval;

- our ability to establish and maintain strategic collaborations, licensing or other arrangements and the financial terms of such agreements;
- any product liability or other lawsuits related to our products;
- the expenses needed to attract and retain skilled personnel;
- the costs associated with being a public company;
- the costs involved in preparing, filing, prosecuting, maintaining, defending and enforcing patent claims, including litigation costs and the outcome of such litigation; and
- the timing, receipt and amount of sales of, or royalties on, future approved products, if any.

If we are unable to successfully raise sufficient additional capital, through future debt or equity financings, product sales, or through strategic and collaborative ventures with third parties, we will not have sufficient cash flows and liquidity to fund our planned business operations. In that event, we may be forced to limit many, if not all, of our programs and consider other means of creating value for our stockholders, such as licensing to others the development and commercialization of products that we consider valuable and would otherwise likely develop ourselves. If we are unable to raise the necessary capital, we may be forced to curtail all of our activities and, ultimately, potentially cease operations. Even if we are able to raise additional capital, such financings may only be available on unattractive terms, or could result in significant dilution of stockholders' interests. The condensed consolidated financial statements do not include any adjustments relating to recoverability and classification of recorded asset amounts or the amounts and classification of liabilities that might be necessary should we be unable to continue in existence.

Please see "Risk Factors" for additional risks associated with our substantial capital requirements.

Contractual Obligations and Commitments

The following is a summary of our long-term contractual cash obligations as of December 31, 2013:

	Total	Less than one year	1 - 3 Years	3 - 5 Years	More than 5 Years
Operating lease obligations	\$ 284,000	\$ 245,000	\$ 39,000	\$ —	\$ —
Total contractual obligations	\$ 284,000	\$ 245,000	\$ 39,000	\$ —	\$ —

Purchase Commitments

We have no material non-cancelable purchase commitments with contract manufacturers or service providers as we have generally contracted on a cancelable purchase order basis.

Milestone, Royalty-Based and Other Commitments

Under our license agreement with Temple to develop, manufacture, market and sell rigosertib related compounds and derivatives, we are obligated to pay annual license maintenance payments, as well as 25% of any sublicensing fees we receive. We are also required to pay a low-single digit percentage of our net sales of rigosertib as a royalty. During the year ended December 31, 2011, in connection with the execution of the SymBio agreement, we made a payment to Temple in the amount of \$1.9 million. During the year ended December 31, 2012, in connection with the execution of the Baxter agreement, we became obligated to make a payment to Temple in the amount of \$12.5 million. Both of these payments were recorded as research and development expenses.

In May 2010, we entered into an agreement with LLS under which we were to conduct research in return for milestone payments, up to \$10.0 million through 2013. This milestone payment amount was

subsequently reduced to \$8.0 million pursuant to an amendment signed in January 2013. In the event that the research is successful, we must proceed with commercialization of the product or repay the amount funded. In addition, we will owe to LLS regulatory and commercial milestone payments and royalties based on net sales of the product not to exceed three times the aggregate amount funded, or \$24.0 million. During the year ended December 31, 2012, in connection with the execution of the Baxter agreement, we paid \$1.0 million to LLS and we have recorded this amount in research and development expenses. This payment reduced the maximum contingent payment obligation under this agreement to \$23.0 million at December 31, 2012, and there were no changes during the year ended December 31, 2013.

Because the achievement and timing of these milestones and net sales is not fixed and determinable, our commitments under these agreements have not been included on our consolidated balance sheets or in the Contractual Obligations table above.

Off-Balance Sheet Arrangements

We do not have any off-balance sheet arrangements, as defined by applicable SEC regulations.

Segment Reporting

We view our operations and manage our business in one segment, which is the identification and development of oncology therapeutics.

Recent Accounting Pronouncements

In February 2013, the Financial Accounting Standards Board (the "FASB") amended its guidance to require an entity to present the effect of certain significant reclassifications out of accumulated other comprehensive income on the respective line items in net income. The new accounting guidance does not change the items that must be reported in other comprehensive income or when an item of other comprehensive income must be reclassified to net income. The guidance is effective prospectively for fiscal years beginning after December 15, 2012. The Company adopted these new provisions for the quarter beginning January 1, 2013. As the guidance requires additional presentation only, there was no impact to the Company's consolidated financial position, results of operations or cash flows.

In July 2013, the FASB issued guidance clarifying that an unrecognized tax benefit, or a portion of an unrecognized tax benefit, should be presented in the financial statements as a reduction to a deferred tax asset for a net operating loss carryforward, a similar tax loss, or a tax credit carryforward if such settlement is required or expected in the event the uncertain tax benefit is disallowed. In situations where a net operating loss carryforward, a similar tax loss, or a tax credit carryforward is not available at the reporting date under the tax law of the applicable jurisdiction or the tax law of the jurisdiction does not require, and the entity does not intend to use, the deferred tax asset for such purpose, the unrecognized tax benefit should be presented in the financial statements as a liability and should not be netted with the deferred tax asset. The guidance is effective for fiscal years, and interim periods within those years, beginning after December 15, 2013. Early adoption is permitted. The guidance is to be applied prospectively to all unrecognized tax benefits that exist at the effective date. Retrospective application is permitted. The Company is currently evaluating the impact that adoption will have on the determination or reporting of its financial results.

ITEM 7A. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

We are exposed to market risks in the ordinary course of our business. These market risks are principally limited to interest rate fluctuations.

We had cash, cash equivalents and marketable securities of \$100.0 million at December 31, 2013, consisting primarily of funds in cash, money market accounts and US treasury notes. The primary objective of our investment activities is to preserve principal and liquidity while maximizing income without significantly increasing risk. We do not enter into investments for trading or speculative purposes. Due to the short-term nature of our investment portfolio, we do not believe an immediate 10% increase or decrease in interest rates would have a material effect on the fair market value of our portfolio, and accordingly we do not expect our operating results or cash flows to be materially affected by a sudden change in market interest rates.

ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

The financial statements and supplementary data required by this item are listed in Item 15—"Exhibits and Financial Statement Schedules" of this Annual Report.

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

None.

ITEM 9A. CONTROLS AND PROCEDURES

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and our Chief Financial Officer, evaluated the effectiveness of our disclosure controls and procedures as of December 31, 2013. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended (the "Exchange Act"), means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company's management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure. Based on the evaluation of our disclosure controls and procedures as of December 31, 2013, our Chief Executive Officer and Chief Financial Officer concluded that, as of such date, disclosure controls and procedures were not effective at the reasonable assurance level because of the material weakness in internal control over financial reporting described below.

Internal Control Over Financial Reporting

In preparing our consolidated financial statements as of and for the year ended December 31, 2012, we and our independent registered public accounting firm identified control deficiencies in the design and operation of our internal control over financial reporting that together constituted a material weakness in our internal control over financial reporting. A material weakness is a deficiency, or a combination of deficiencies, in internal control over financial reporting such that there is a reasonable possibility that a material misstatement of our financial statements will not be prevented or detected on a timely basis. The material weakness identified was that we did not have sufficient financial reporting and accounting staff with appropriate training in GAAP and SEC rules and

regulations with respect to financial reporting. As such, our controls over financial reporting were not designed or operating effectively, and as a result there were adjustments required in connection with closing our books and records and preparing our 2012 consolidated financial statements.

We have discussed this material weakness with our independent registered public accounting firm and our Audit Committee. To remediate this material weakness, we have expanded our staff by hiring a Chief Financial Officer, a Director of Financial Reporting and a Vice President of Financial Planning and Accounting, each with prior public company financial reporting experience. We have also hired additional finance and accounting personnel with appropriate training to build our financial management and reporting infrastructure. We have initiated processes to further develop and document our accounting policies and financial reporting procedures and to implement additional internal controls. Although, we believe we have made improvements in our disclosure controls and procedures, as of our December 31, 2013 financial closing process we had not yet fully remediated the material weakness discussed above. In addition, we cannot provide assurance that we have identified all of our existing material weaknesses, or that we will not in the future have additional material weaknesses.

Neither we nor our independent registered public accounting firm has performed an evaluation of our internal control over financial reporting during any period in accordance with the provisions of the Sarbanes-Oxley Act. In light of the control deficiencies and the resulting material weakness that were identified as a result of the limited procedures performed, we believe that it is possible that had we and 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 material weaknesses and significant control deficiencies may have been identified. However, for as long as we remain an "emerging growth company" as defined in the JOBS Act, we intend to utilize the provision exempting us from the requirement that our independent registered public accounting firm provide an attestation on the effectiveness of our internal control over financial reporting.

Management's Report on Internal Control Over Financial Reporting

This annual report does not include a report of management's assessment regarding internal control over financial reporting or an attestation report of the company's registered public accounting firm due to a transition period established by rules of the Securities and Exchange Commission for newly public companies.

Changes in Internal Control Over Financial Reporting

There has been no change in our internal control over financial reporting during the last fiscal quarter that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

ITEM 9B. OTHER INFORMATION

None.

PART III

ITEM 10. DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE

Information with respect to this item is set forth in the Proxy Statement for the 2014 Annual Meeting of Stockholders (the "Proxy Statement") under the headings "Election of Directors," "Executive Officers," "Section 16(a) Beneficial Ownership Reporting Compliance," "Code of Ethics" and "Corporate Governance" and is incorporated herein by reference. The Proxy Statement will be filed with the SEC within 120 days after the end of the fiscal year covered by this Annual Report.

ITEM 11. EXECUTIVE COMPENSATION

Information with respect to this item is set forth in the Proxy Statement under the headings "Executive Compensation" and "Director Compensation," and is incorporated herein by reference. The Proxy Statement will be filed with the SEC within 120 days after the end of the fiscal year covered by this Annual Report.

ITEM 12. SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS

Information with respect to this item is set forth in the Proxy Statement under the headings "Security Ownership of Certain Beneficial Owners and Management" and "Executive Compensation," and is incorporated herein by reference. The Proxy Statement will be filed with the SEC within 120 days after the end of the fiscal year covered by this Annual Report.

ITEM 13. CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS, AND DIRECTOR INDEPENDENCE

Information with respect to this item is set forth in the Proxy Statement under the headings "Certain Relationships and Related Party Transactions" and "Corporate Governance" and is incorporated herein by reference. The Proxy Statement will be filed with the SEC within 120 days after the end of the fiscal year covered by this Annual Report.

ITEM 14. PRINCIPAL ACCOUNTING FEES AND SERVICES

Information with respect to this item is set forth in the Proxy Statement under the heading "Ratification of the Selection of Independent Registered Public Accounting Firm," and is incorporated herein by reference. The Proxy Statement will be filed with the SEC within 120 days after the end of the fiscal year covered by this Annual Report.

PART IV

ITEM 15. EXHIBITS AND FINANCIAL STATEMENT SCHEDULES

- (a) (1) Financial Statements: See Index to Consolidated Financial Statements on page F-1.
- (3) Exhibits: See Exhibits Index on pages 80 to 82.

<u>Signature</u>	<u>Title</u>	<u>Date</u>
<u>/s/ E. PREMKUMAR REDDY, PH.D.</u> E. Premkumar Reddy, Ph.D.	Director	March 20, 2014
<u>/s/ ANNE M. VANLENT</u> Anne M. VanLent	Director	March 20, 2014

Exhibits Index

Exhibit Number	Exhibit Description
3.1	Tenth Amended and Restated Certificate of Incorporation of Onconova Therapeutics, Inc. <i>(Incorporated by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K filed on July 25, 2013).</i>
3.2	Amended and Restated Bylaws of Onconova Therapeutics, Inc. <i>(Incorporated by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K filed on July 25, 2013).</i>
4.1	Form of Certificate of Common Stock <i>(Incorporated by reference to Exhibit 4.1 to Pre-Effective Amendment No. 1 the Company's Registration Statement on Form S-1 filed on July 11, 2013.)</i>
4.2	Eighth Amended and Restated Stockholders' Agreement, effective as of July 27, 2012, by and among Onconova Therapeutics, Inc. and certain stockholders named therein <i>(Incorporated by reference to Exhibit 4.2 to Pre-Effective Amendment No. 1 to the Company's Registration Statement on Form S-1 filed on July 11, 2013).</i>
4.3	Amendment No. 1 to Eighth Amended and Restated Stockholders' Agreement, effective as of July 9, 2013 <i>(Incorporated by reference to Exhibit 4.2 to Pre-Effective Amendment No. 1 the Company's Registration Statement on Form S-1 filed on July 11, 2013).</i>
10.1*	Development and License Agreement, effective as of September 19, 2012, by and between Onconova Therapeutics, Inc. and Baxter Healthcare SA <i>(Incorporated by reference to Exhibit 10.1 to Pre-Effective Amendment No. 2 the Company's Registration Statement on Form S-1 filed on July 18, 2013).</i>
10.2*	License Agreement, effective as of July 5, 2011, by and between Onconova Therapeutics, Inc. and SymBio Pharmaceuticals Limited <i>(Incorporated by reference to Exhibit 10.2 to Pre-Effective Amendment No. 2 the Company's Registration Statement on Form S-1 filed on July 18, 2013).</i>
10.3*	First Amendment to License Agreement, effective as of September 2, 2011, by and between Onconova Therapeutics, Inc. and SymBio Pharmaceuticals Limited <i>(Incorporated by reference to Exhibit 10.3 to the Company's Registration Statement on Form S-1 filed on June 14, 2013).</i>
10.4*	License Agreement, effective as of January 1, 1999, by and between Onconova Therapeutics, Inc. and Temple University—Of The Commonwealth System of Higher Education <i>(Incorporated by reference to Exhibit 10.4 to the Company's Registration Statement on Form S-1 filed on June 14, 2013).</i>
10.5*	Amendment to License Agreement, effective as of September 1, 2000, by and between Temple University—Of The Commonwealth System of Higher Education and Onconova Therapeutics, Inc. <i>(Incorporated by reference to Exhibit 10.5 to the Company's Registration Statement on Form S-1 filed on June 14, 2013).</i>
10.6*	Amendment #1 to Exclusive License Agreement, effective as of March 21, 2013, by and between Temple University—Of The Commonwealth System of Higher Education and Onconova Therapeutics, Inc. <i>(Incorporated by reference to Exhibit 10.6 to the Company's Registration Statement on Form S-1 filed on June 14, 2013).</i>
10.7*	Definitive Agreement, effective as of May 12, 2010, by and between Onconova Therapeutics, Inc. and The Leukemia and Lymphoma Society <i>(Incorporated by reference to Exhibit 10.7 to the Company's Registration Statement on Form S-1 filed on June 14, 2013).</i>

Exhibit Number	Exhibit Description
10.8*	First Amendment to Definitive Agreement, effective as of June 23, 2011, by and between Onconova Therapeutics, Inc. and The Leukemia and Lymphoma Society (<i>Incorporated by reference to Exhibit 10.8 to the Company's Registration Statement on Form S-1 filed on June 14, 2013</i>).
10.9*	Second Amendment to Definitive Agreement, effective as of May 29, 2012, by and between Onconova Therapeutics, Inc. and The Leukemia and Lymphoma Society (<i>Incorporated by reference to Exhibit 10.9 to the Company's Registration Statement on Form S-1 filed on June 14, 2013</i>).
10.10*	Third Amendment to Definitive Agreement, effective as of January 5, 2013, by and between Onconova Therapeutics, Inc. and The Leukemia and Lymphoma Society (<i>Incorporated by reference to Exhibit 10.10 to the Company's Registration Statement on Form S-1 filed on June 14, 2013</i>).
10.11	Termination of Agreement, effective as of February 5, 2013, by and between Onconova Therapeutics, Inc. and The Leukemia and Lymphoma Society (<i>Incorporated by reference to Exhibit 10.11 to the Company's Registration Statement on Form S-1 filed on June 14, 2013</i>).
10.12*	Limited Liability Company Agreement of GBO, LLC, dated as of December 12, 2012, by and between Onconova Therapeutics, Inc. and GVK Biosciences Private Limited (<i>Incorporated by reference to Exhibit 10.12 to the Company's Registration Statement on Form S-1 filed on June 14, 2013</i>).
10.13+	Onconova Therapeutics, Inc. 2007 Equity Compensation Plan, and forms of agreement thereunder (<i>Incorporated by reference to Exhibit 10.13 to Pre-Effective Amendment No. 1 the Company's Registration Statement on Form S-1 filed on July 11, 2013</i>).
10.14+	Employment Agreement, effective as of April 1, 2007, by and between Onconova Therapeutics, Inc. and Ramesh Kumar, Ph.D., including extension letter, dated April 10, 2010, and Employment Agreement Renewal, dated January 10, 2013 (<i>Incorporated by reference to Exhibit 10.14 to the Company's Registration Statement on Form S-1 filed on June 14, 2013</i>).
10.15+	Amendment to Employment Agreement, effective as of December 21, 2012, by and between Onconova Therapeutics, Inc. and Ramesh Kumar, Ph.D. (<i>Incorporated by reference to Exhibit 10.15 to the Company's Registration Statement on Form S-1 filed on June 14, 2013</i>).
10.16+	Employment Agreement, effective as of September 1, 2012, by and between Onconova Therapeutics, Inc. and Thomas McKearn, M.D., Ph.D. (<i>Incorporated by reference to Exhibit 10.16 to the Company's Registration Statement on Form S-1 filed on June 14, 2013</i>).
10.17+	Amendment to Employment Agreement, effective as of April 9, 2013, by and between Onconova Therapeutics, Inc. and Thomas McKearn, M.D., Ph.D. (<i>Incorporated by reference to Exhibit 10.17 to the Company's Registration Statement on Form S-1 filed on June 14, 2013</i>).
10.18+	Employment Agreement, effective as of April 17, 2008, by and between Onconova Therapeutics, Inc. and François Wilhelm, M.D., including Employment Agreement Renewals, dated March 30, 2010 and January 10, 2013 (<i>Incorporated by reference to Exhibit 10.18 to the Company's Registration Statement on Form S-1 filed on June 14, 2013</i>).

<u>Exhibit Number</u>	<u>Exhibit Description</u>
10.19+	Amendment to Employment Agreement, effective as of December 21, 2012, by and between Onconova Therapeutics, Inc. and Francois Wilhelm, M.D. <i>(Incorporated by reference to Exhibit 10.19 to the Company's Registration Statement on Form S-1 filed on June 14, 2013).</i>
10.20+	Employment Agreement, effective as of January 1, 2007, by and between Onconova Therapeutics, Inc. and Dr. Manoj Maniar, including Employment Agreement Renewals, dated March 30, 2010 and January 10, 2013 <i>(Incorporated by reference to Exhibit 10.20 to the Company's Registration Statement on Form S-1 filed on June 14, 2013).</i>
10.21+	Amendment to Employment Agreement, effective as of December 21, 2012, by and between Onconova Therapeutics, Inc. and Dr. Manoj Maniar <i>(Incorporated by reference to Exhibit 10.21 to the Company's Registration Statement on Form S-1 filed on June 14, 2013).</i>
10.22+	Employment Agreement, effective as of March 20, 2013, by and between Onconova Therapeutics, Inc. and Ajay Bansal <i>(Incorporated by reference to Exhibit 10.22 to the Company's Registration Statement on Form S-1 filed on June 14, 2013).</i>
10.23+	Consulting Agreement, effective as of January 1, 2012, by and between Onconova Therapeutics, Inc. and E. Premkumar Reddy, Ph.D., including Consultant Agreement Renewal, dated February 27, 2013 <i>(Incorporated by reference to Exhibit 10.23 to the Company's Registration Statement on Form S-1 filed on June 14, 2013).</i>
10.24+	Form of Indemnification Agreement entered into by and between Onconova Therapeutics, Inc. and [each](1) director and executive officer <i>(Incorporated by reference to Exhibit 10.24 to Pre-Effective Amendment No. 1 the Company's Registration Statement on Form S-1 filed on July 11, 2013).</i>
10.25+	Onconova Therapeutics, Inc. 2013 Equity Compensation Plan, and forms of agreement thereunder <i>(Incorporated by reference to Exhibit 10.25 to Pre-Effective Amendment No. 1 the Company's Registration Statement on Form S-1 filed on July 11, 2013).</i>
10.26+	Onconova Therapeutics, Inc. 2013 Performance Bonus Plan <i>(Incorporated by reference to Exhibit 10.26 to Pre-Effective Amendment No. 1 the Company's Registration Statement on Form S-1 filed on July 11, 2013).</i>
21.1	Subsidiaries of Onconova Therapeutics, Inc.
23.1	Consent of Ernst & Young, LLP.
23.2	Consent of Eisner Amper, LLP.
31.1	Certification of Principal Executive Officer pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2	Certification of Principal Financial Officer pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1	Certification of Principal Executive Officer pursuant to 18 U.S.C. Section 1350 as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
32.2	Certification of Principal Financial Officer pursuant to 18 U.S.C. Section 1350 as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101.INS	XBRL Instance
101.SCH	XBRL Taxonomy Extension Schema Document

<u>Exhibit Number</u>	<u>Exhibit Description</u>
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	XBRL Taxonomy Extension Calculation Linkbase Document
101.LAB	XBRL Taxonomy Extension Labels Linkbase Document
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document

+ Indicates management contract or compensatory plan.

* Confidential treatment has been requested with respect to certain portions of this exhibit. Omitted portions have been filed separately with the Securities and Exchange Commission.

ONCONOVA THERAPEUTICS, INC. AND SUBSIDIARIES

Index to Consolidated Financial Statements

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Report of Independent Registered Public Accounting Firm

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

We have audited the accompanying consolidated balance sheets of Onconova Therapeutics, Inc. as of December 31, 2013 and 2012, and the related consolidated statements of operations, comprehensive loss, redeemable convertible preferred stock and stockholders' equity, and cash flows for the years ended December 31, 2013 and 2012. These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audits.

We conducted our audits in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. We were not engaged to perform an audit of the Company's internal control over financial reporting. Our audits included consideration of internal control over financial reporting as a basis for designing audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion. An audit also includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements, assessing the accounting principles used and significant estimates made by management, and evaluating the overall consolidated financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the financial statements referred to above present fairly, in all material respects, the consolidated financial position of Onconova Therapeutics, Inc. at December 31, 2013 and 2012, and the consolidated results of its operations and its cash flows for the years ended December 31, 2013 and 2012, in conformity with U.S. generally accepted accounting principles.

/s/ Ernst & Young LLP

Philadelphia, Pennsylvania
March 20, 2014

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

The Board of Directors and Stockholders
Onconova Therapeutics, Inc.

We have audited the statements of operations and comprehensive loss, redeemable convertible preferred stock and stockholders' deficit, and cash flows for the year ended December 31, 2011. The financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audit.

We conducted our audit in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. Our audit included consideration of internal control over financial reporting as a basis for designing audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion. An audit includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements. An audit also includes assessing the accounting principles used and significant estimates made by management, as well as evaluating the overall financial statement presentation. We believe that our audit provides a reasonable basis for our opinion.

In our opinion, the financial statements referred to above present fairly, in all material respects, the results of the Company's operations and its cash flows for the year ended December 31, 2011, in conformity with accounting principles generally accepted in the United States of America.

/s/ EISNERAMPER LLP

Iselin, New Jersey
May 2, 2013, except for the third paragraph of Note 18, as to which the date is July 17, 2013

Onconova Therapeutics, Inc.**Consolidated Balance Sheets**

	<u>December 31,</u>	
	<u>2013</u>	<u>2012</u>
Assets		
Current assets:		
Cash and cash equivalents	\$ 60,009,000	\$ 81,527,000
Marketable securities	39,994,000	—
Prepaid expenses and other current assets	4,387,000	1,725,000
Total current assets	104,390,000	83,252,000
Property and equipment, net	626,000	463,000
Restricted cash	125,000	125,000
Other non-current assets	12,000	12,000
Total assets	<u>\$ 105,153,000</u>	<u>\$ 83,852,000</u>
Liabilities, redeemable convertible preferred stock and stockholders' equity (deficit)		
Current liabilities:		
Accounts payable	\$ 3,710,000	\$ 5,517,000
Accrued expenses and other current liabilities	5,820,000	3,925,000
Warrant liability	20,000	62,000
Option liability	—	11,967,000
Deferred revenue	788,000	3,907,000
Total current liabilities	10,338,000	25,378,000
Deferred revenue, non-current	13,909,000	15,421,000
Other	6,000	44,000
Total liabilities	<u>24,253,000</u>	<u>40,843,000</u>
Commitments and contingencies		
Redeemable convertible preferred stock, \$0.01 par value per share, none and 18,548,253 shares authorized at December 31, 2013 and 2012, none and 16,912,199 issued and outstanding at December 31, 2013 and 2012	—	201,315,000
Stockholders' equity (deficit):		
Preferred stock, \$0.01 par value, 5,000,000 and none authorized at December 31, 2013 and 2012, none issued and outstanding at December 31, 2013 and 2012	—	—
Common stock, \$0.01 par value, 75,000,000 and 30,145,155 authorized at December 31, 2013 and 2012, 21,467,482 and 2,606,484 shares issued and outstanding at December 31 2013 and 2012	215,000	26,000
Additional paid in capital	311,093,000	10,021,000
Accumulated other comprehensive income	1,000	—
Accumulated deficit	(230,896,000)	(168,353,000)
Total Onconova Therapeutics, Inc. stockholders' equity (deficit)	80,413,000	(158,306,000)
Non-controlling interest	487,000	—
Total stockholders' equity (deficit)	<u>80,900,000</u>	<u>(158,306,000)</u>
Total liabilities, redeemable convertible preferred stock and stockholders' equity (deficit)	<u>\$ 105,153,000</u>	<u>\$ 83,852,000</u>

See accompanying notes to consolidated financial statements.

Onconova Therapeutics, Inc.**Consolidated Statements of Operations**

	Years ended December 31,		
	2013	2012	2011
Revenue	\$ 4,753,000	\$ 46,190,000	\$ 1,487,000
Operating expenses:			
General and administrative	16,793,000	15,707,000	6,436,000
Research and development	50,182,000	52,762,000	22,624,000
Total operating expenses	66,975,000	68,469,000	29,060,000
Loss from operations	(62,222,000)	(22,279,000)	(27,573,000)
Change in fair value of warrant liability	42,000	367,000	1,287,000
Interest expense	(4,000)	(8,608,000)	(19,000)
Other income, net	63,000	608,000	11,000
Net loss before income taxes	(62,121,000)	(29,912,000)	(26,294,000)
Income taxes	435,000	—	—
Net loss	(62,556,000)	(29,912,000)	(26,294,000)
Net loss attributable to non-controlling interest	13,000	—	—
Net loss attributable to Onconova Therapeutics, Inc	(62,543,000)	(29,912,000)	(26,294,000)
Accretion of redeemable convertible preferred stock	(2,320,000)	(3,953,000)	(4,020,000)
Net loss applicable to common stockholders	\$ (64,863,000)	\$ (33,865,000)	\$ (30,314,000)
Net loss per share of common stock, basic and diluted	\$ (6.12)	\$ (15.35)	\$ (14.18)
Basic and diluted weighted average shares outstanding	10,594,227	2,206,888	2,137,403

See accompanying notes to consolidated financial statements.

Onconova Therapeutics, Inc.**Consolidated Statements of Comprehensive Loss**

	<u>Years ended December 31,</u>		
	<u>2013</u>	<u>2012</u>	<u>2011</u>
Net loss	\$ (62,556,000)	\$ (29,912,000)	\$ (26,294,000)
Other comprehensive income, before tax:			
Foreign currency translation adjustments, net	1,000	—	—
Other comprehensive income, net of tax	1,000	—	—
Comprehensive loss	<u>(62,555,000)</u>	<u>(29,912,000)</u>	<u>(26,294,000)</u>
Comprehensive loss attributable to non-controlling interest	13,000	—	—
Comprehensive loss attributable to Onconova Therapeutics, Inc	<u>\$ (62,542,000)</u>	<u>\$ (29,912,000)</u>	<u>\$ (26,294,000)</u>

See accompanying notes to consolidated financial statements.

Onconova Therapeutics, Inc.
Consolidated Statements of Redeemable Convertible Preferred Stock and Stockholders' Equity

	Redeemable Convertible Preferred Stock			Stockholders' Equity (Deficit)						
	Shares	Amount	Preferred Stock Subscribed	Common Stock		Additional Paid in Capital	Accumulated deficit	Accumulated other comprehensive income	Non- controlling interest	Total
				Shares	Amount					
Balance at January 1, 2011	10,211,835	\$ 105,734,000	\$ 300,000	2,068,032	\$ 21,000	\$ 7,000	\$(108,745,000)	\$ —	\$ —	\$(108,717,000)
Net loss	—	—	—	—	—	—	(26,294,000)	—	—	(26,294,000)
Issuance of preferred stock, net of issuance costs	819,329	7,917,000	(300,000)	—	—	—	—	—	—	—
Exercise of stock options	—	—	—	99,896	1,000	611,000	—	—	—	612,000
Issuance of preferred stock upon exercise of warrants	196,005	2,326,000	—	—	—	—	—	—	—	—
Accretion of preferred stock to redemption value	—	4,020,000	—	—	—	(618,000)	(3,402,000)	—	—	(4,020,000)
Balance at December 31, 2011	<u>11,227,169</u>	<u>119,997,000</u>	<u>—</u>	<u>2,167,928</u>	<u>22,000</u>	<u>—</u>	<u>(138,441,000)</u>	<u>—</u>	<u>—</u>	<u>(138,419,000)</u>
Net loss	—	—	—	—	—	—	(29,912,000)	—	—	(29,912,000)
Issuance of preferred stock, net of issuance costs	3,030,303	47,796,000	—	—	—	—	—	—	—	—
Exercise of stock options	—	—	—	438,556	4,000	4,690,000	—	—	—	4,694,000
Proceeds from stockholder in connection with settlement of stock option exercises	—	—	—	—	—	3,943,000	—	—	—	3,943,000
Settlement of stock option liabilities	—	—	—	—	—	(2,835,000)	—	—	—	(2,835,000)
Issuance of preferred stock upon exercise of warrants	221,399	2,802,000	—	—	—	—	—	—	—	—
Exchange of convertible debt and preferred stock	2,433,328	26,767,000	—	—	—	—	—	—	—	—
Beneficial conversion feature on convertible debt	—	—	—	—	—	8,176,000	—	—	—	8,176,000
Accretion of preferred stock to redemption value	—	3,953,000	—	—	—	(3,953,000)	—	—	—	(3,953,000)
Balance at December 31, 2012	<u>16,912,199</u>	<u>201,315,000</u>	<u>—</u>	<u>2,606,484</u>	<u>26,000</u>	<u>10,021,000</u>	<u>(168,353,000)</u>	<u>—</u>	<u>—</u>	<u>(158,306,000)</u>
Net loss	—	—	—	—	—	—	(62,543,000)	—	(13,000)	(62,556,000)
Contribution from non-controlling interest	—	—	—	—	—	—	—	—	500,000	500,000
Other comprehensive income	—	—	—	—	—	—	—	1,000	—	1,000
Exercise of stock options	—	—	—	81,204	1,000	194,000	—	—	—	195,000
Stock-based compensation	—	—	—	—	—	5,462,000	—	—	—	5,462,000
Reclassification of stock option liability	—	—	—	—	—	14,482,000	—	—	—	14,482,000
Accretion of preferred stock to redemption value	—	2,320,000	—	—	—	(2,320,000)	—	—	—	(2,320,000)
Conversion convertible preferred stock into common stock	(16,912,199)	(203,635,000)	—	12,838,127	129,000	203,502,000	—	—	—	203,631,000
Issuance of common stock, net of issuance costs	—	—	—	5,941,667	59,000	79,752,000	—	—	—	79,811,000
Balance at December 31, 2013	<u>—</u>	<u>\$ —</u>	<u>\$ —</u>	<u>21,467,482</u>	<u>\$215,000</u>	<u>\$311,093,000</u>	<u>\$(230,896,000)</u>	<u>\$ 1,000</u>	<u>\$ 487,000</u>	<u>\$ 80,900,000</u>

See accompanying notes to consolidated financial statements.



Onconova Therapeutics, Inc.
Consolidated Statements of Cash Flows

	Year Ended December 31,		
	2013	2012	2011
Operating activities:			
Net loss	\$ (62,556,000)	\$ (29,912,000)	\$ (26,294,000)
Adjustment to reconcile net loss to net cash (used in) provided by operating activities:			
Depreciation and amortization	446,000	319,000	316,000
Loss on asset disposal	—	3,000	—
Amortization of deferred financing fees	—	15,000	21,000
Amortization of debt discount	—	8,176,000	—
Change in fair value of warrant liabilities	(42,000)	(367,000)	(1,287,000)
Treasury note discount amortization	(4,000)	—	—
Stock compensation expense	8,015,000	13,844,000	6,000
Changes in assets and liabilities:			
Grants receivable	—	78,000	1,730,000
Prepaid expenses and other current assets	(2,662,000)	(1,098,000)	253,000
Other assets	—	(15,000)	—
Accounts payable	(1,807,000)	(97,000)	2,230,000
Accrued expenses	1,894,000	2,573,000	1,123,000
Other liabilities	(37,000)	(41,000)	58,000
Deferred revenue	(4,631,000)	8,155,000	7,673,000
Net cash (used in) provided by operating activities	<u>(61,384,000)</u>	<u>1,633,000</u>	<u>(14,171,000)</u>
Investing activities:			
Payments for purchase of property and equipment	(609,000)	(279,000)	(256,000)
Security deposits	—	—	15,000
Purchases of marketable securities	(39,990,000)	—	—
Net cash used in investing activities	<u>(40,599,000)</u>	<u>(279,000)</u>	<u>(241,000)</u>
Financing activities:			
Proceeds from initial public offering of common stock, net of issuance costs	79,811,000	—	—
Proceeds from the exercise of stock options	157,000	165,000	154,000
Contribution from non-controlling interest	500,000	—	—
Reverse stock split cash paid in lieu of fractional shares	(4,000)	—	—
Proceeds from stockholder in connection with settlement of stock option exercises	—	3,943,000	—
Settlement of stock options	—	(2,835,000)	—
Proceeds from the exercise of warrants	—	2,167,000	1,918,000
Proceeds from the sale of Series H preferred stock	—	400,000	7,218,000
Proceeds from the sale of Series J preferred stock	—	47,796,000	—
Repayments of long-term debt	—	—	(917,000)
Release of cash restricted for debt repayment	—	—	792,000
Proceeds from stockholder loan and convertible debt	—	25,824,000	620,000
Net cash provided by financing activities	<u>80,464,000</u>	<u>77,460,000</u>	<u>9,785,000</u>
Effect of foreign currency translation on cash	1,000	—	—
Net (decrease) increase in cash and cash equivalents	<u>(21,518,000)</u>	<u>78,814,000</u>	<u>(4,627,000)</u>
Cash and cash equivalents at beginning of period	81,527,000	2,713,000	7,340,000
Cash and cash equivalents at end of period	<u>\$ 60,009,000</u>	<u>\$ 81,527,000</u>	<u>\$ 2,713,000</u>
Supplemental disclosure of cash flow information:			
Income taxes paid	<u>\$ 435,000</u>	<u>—</u>	<u>—</u>

See accompanying notes to consolidated financial statements.

Onconova Therapeutics, Inc.

Notes to Consolidated Financial Statements

1. Nature of Business

The Company

Onconova Therapeutics, Inc. (the "Company") was incorporated in the State of Delaware on December 22, 1998 and commenced operations on January 1, 1999. The Company's headquarters are located in Newtown, Pennsylvania. The Company is a clinical-stage biopharmaceutical company focused on discovering and developing novel small molecule drug candidates to treat cancer. Using its proprietary chemistry platform, the Company has created an extensive library of targeted anti-cancer agents designed to work against specific cellular pathways that are important to cancer cells. The Company believes that the drug candidates in its pipeline have the potential to be efficacious in a wide variety of cancers without causing harm to normal cells. The Company has three clinical-stage product candidates and six preclinical programs. To accelerate and broaden the development of rigosertib, the Company's most advanced product candidate, the Company entered into a collaboration and license agreement in 2012 with Baxter Healthcare SA ("Baxter"), a subsidiary of Baxter International Inc., to commercialize rigosertib in Europe. In 2011, the Company entered into a collaboration and license agreement with Symbio Pharmaceuticals Limited ("Symbio") to commercialize rigosertib in Japan and Korea. The Company has retained development and commercialization rights to rigosertib in the rest of the world, including the United States. During 2012, Onconova Europe GmbH was established as a wholly owned subsidiary of the Company for the purpose of further developing business in Europe. In April 2013, GBO, LLC, a Delaware limited liability company, ("GBO") was formed pursuant to a collaboration agreement with GVK Biosciences Private Limited, a private limited company located in India, ("GVK BIO") to collaborate and develop two new clinical programs using the Company's technology platform.

Liquidity

The Company has incurred recurring operating losses since inception. For the year ended December 31, 2013, the Company incurred a net loss of \$62,556,000 and as of December 31, 2013, the Company had generated an accumulated deficit of \$230,896,000. The Company anticipates operating losses to continue for the foreseeable future due to, among other things, costs related to research funding, development of its product candidates and its preclinical programs, strategic alliances and the development of its administrative organization. The Company will require substantial additional financing to fund its operations and to continue to execute its strategy.

The Company raised significant capital through the issuance of its redeemable convertible preferred stock, par value \$0.01 per share, in ten series denominated as Series A through Series J ("Series A Preferred Stock" through "Series J Preferred Stock," respectively, and collectively the "Preferred Stock"). See Note 10.

On July 30, 2013, the Company completed its initial public offering (the "IPO") of 5,941,667 shares of the Company's common stock, par value \$0.01 per share ("Common Stock"), at a price of \$15.00 per share, including 775,000 shares of Common Stock issued upon the exercise in full by the underwriters of their option to purchase additional shares at the same price to cover over-allotments. The Company received net proceeds of \$79,811,000 from the sale, net of underwriting discounts and commissions and other estimated offering expenses. Immediately prior to the consummation of the IPO, all outstanding shares of Preferred Stock automatically converted into shares of Common Stock at the applicable conversion ratio then in effect. As a result of the conversion, as of July 30, 2013, the Company had no shares of Preferred Stock outstanding. See Note 10.

Onconova Therapeutics, Inc.

Notes to Consolidated Financial Statements (Continued)

2. Summary of Significant Accounting Policies

Basis of Presentation

The consolidated financial statements are prepared in conformity with accounting principles generally accepted in the United States ("GAAP"). The financial statements include the consolidated accounts of the Company, its wholly-owned subsidiary, Onconova Europe GmbH and GBO. All significant intercompany transactions have been eliminated.

Segment Information

Operating segments are defined as components of an enterprise about which separate discrete information is available for evaluation by the chief operating decision maker, or decision-making group, in deciding how to allocate resources and in assessing performance. The Company views its operations and manages its business in one segment, which is the identification and development of oncology therapeutics.

Use of Estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets, liabilities, revenues, expenses, other comprehensive income and related disclosures. On an ongoing basis, management evaluates its estimates, including estimates related to clinical trial accruals, warrant liability, and allocation of consideration to multiple element collaborative arrangements. The Company bases its estimates on historical experience and other market-specific or other relevant assumptions that it believes to be reasonable under the circumstances. Actual results may differ from those estimates or assumptions.

Prior to completion of its IPO on July 30, 2013, the Company utilized estimates and assumptions in determining the fair value of its Common Stock. The Company granted stock options at exercise prices not less than the fair value of its Common Stock as determined by the board of directors, with input from management. Management used the assistance of a third-party valuation firm in estimating the fair value of the Common Stock. The board of directors determined the estimated fair value of the Common Stock based on a number of objective and subjective factors, including external market conditions affecting the biotechnology industry sector and the historic prices at which the Company sold shares of Preferred Stock.

Concentrations of Credit Risk and Off-Balance Sheet Risk

Financial instruments that potentially subject the Company to concentrations of credit risk are primarily cash, cash equivalents, restricted cash and marketable securities. The Company maintains a portion of its cash and cash equivalent balances in the form of money market accounts with financial institutions that management believes are creditworthy. Marketable securities are invested in U.S. Treasury obligations. The Company has no financial instruments with off-balance sheet risk of loss.

Cash and Cash Equivalents

The Company considers all highly liquid investments with original or remaining maturity from the date of purchase of three months or less to be cash equivalents. Cash and cash equivalents include bank demand deposits, marketable securities with maturities of three months or less at purchase, and

Onconova Therapeutics, Inc.**Notes to Consolidated Financial Statements (Continued)****2. Summary of Significant Accounting Policies (Continued)**

money market funds that invest primarily in certificates of deposit, commercial paper and U.S. government and U.S. government agency obligations. Cash equivalents are reported at fair value.

Marketable Securities

Marketable securities with original maturities longer than three months but which mature in less than one year from the balance sheet date are classified as current assets. Marketable securities that mature more than one year from the balance sheet date are classified as noncurrent assets. Marketable securities that the Company has the intent and ability to hold to maturity are classified as investments held-to-maturity and are reported at amortized cost. The difference between the acquisition cost and face values of held-to-maturity investments is amortized over the remaining term of the investments and added to or subtracted from the acquisition cost and interest income. As of December 31, 2013, all of the Company's investments were classified as held-to-maturity.

Fair Value of Financial Instruments

The carrying amounts reported in the accompanying consolidated financial statements for cash and cash equivalents, marketable securities, accounts payable and accrued liabilities approximate their respective fair values because of the short-term nature of these accounts. The fair value of the warrant liability is discussed in Note 4, "Fair Value Measurements."

Property and Equipment

Property and equipment are stated at cost, less accumulated depreciation. Property and equipment are depreciated using the straight-line method over the estimated useful lives of the assets. Leasehold improvements are amortized over the useful life of the asset or the lease term, whichever is shorter. Maintenance and repairs are expensed as incurred. The following estimated useful lives were used to depreciate the Company's assets:

	<u>Estimated Useful Life</u>
Lab equipment	5 - 6 years
Software	3 years
Computer and office equipment	5 - 6 years
Leasehold improvements	Shorter of the lease term or estimated useful life

Upon retirement or sale, the cost of the disposed asset and the related accumulated depreciation are removed from the accounts and any resulting gain or loss is recognized.

The Company reviews long-lived assets for impairment when events or changes in circumstances indicate that the carrying value of the assets may not be recoverable. Recoverability is measured by comparison of the assets' book value to future net undiscounted cash flows that the assets are expected to generate. If such assets are considered to be impaired, the impairment to be recognized is measured by the amount by which the book value of the assets exceeds their fair value, which is measured based on the projected discounted future net cash flows generated from the assets. No impairment losses have been recorded through December 31, 2013.

Onconova Therapeutics, Inc.

Notes to Consolidated Financial Statements (Continued)

2. Summary of Significant Accounting Policies (Continued)

Restricted Cash

Under the Company's office lease, the Company is required to provide the landlord a \$125,000 letter of credit, which is recorded as restricted cash on the consolidated balance sheets as of December 31, 2013 and 2012.

Foreign Currency Translation

The reporting currency of the Company and its U.S. subsidiaries is the U.S. dollar. The functional currency of the Company's non-U.S. subsidiary is the local currency. Assets and liabilities of the foreign subsidiary are translated into U.S. dollars based on exchange rates at the end of the period. Revenues and expenses are translated at average exchange rates during the reporting period. Gains and losses arising from the translation of assets and liabilities are included as a component of accumulated other comprehensive income. Gains and losses resulting from foreign currency transactions are reflected within the Company's results of operations. The Company has not utilized any foreign currency hedging strategies to mitigate the effect of its foreign currency exposure.

Revenue Recognition

Currently, the Company's revenue is generated primarily through collaborative research and license agreements. The terms of these agreements contain multiple deliverables which may include (i) licenses, (ii) research and development activities, (iii) participation in joint steering committees and (iv) product supply. The terms of these agreements may include nonrefundable upfront license fees, payments for research and development activities, payments based upon the achievement of certain milestones, royalty payments based on product sales derived from the collaboration, and payments for supplying product. In all instances, revenue is recognized only when the price is fixed or determinable, persuasive evidence of an arrangement exists, delivery has occurred or the services have been rendered, collectability of the resulting receivable is reasonably assured, and the Company has fulfilled its performance obligations under the contract.

For arrangements with multiple elements, the Company recognizes revenue in accordance with the Financial Accounting Standards Board ("FASB") Accounting Standards Update ("ASU") No. 2009-13, *Multiple-Deliverable Revenue Arrangements* ("ASU 2009-13"), which provides guidance for separating and allocating consideration in a multiple element arrangement. The selling prices of deliverables under an arrangement may be derived using third-party evidence ("TPE"), or a best estimate of selling price ("BESP"), if vendor-specific objective evidence of selling price ("VSOE") is not available. The objective of BESP is to determine the price at which the Company would transact a sale if the element within the license agreement was sold on a standalone basis. Establishing BESP involves management's judgment and considers multiple factors, including market conditions and company-specific factors, including those factors contemplated in negotiating the agreements, as well as internally developed models that include assumptions related to market opportunity, discounted cash flows, estimated development costs, probability of success and the time needed to commercialize a product candidate pursuant to the license. In validating the BESP, management considers whether changes in key assumptions used to determine the BESP will have a significant effect on the allocation of the arrangement consideration between the multiple deliverables. The Company may use third-party valuation specialists to assist it in determining BESP. Deliverables under the arrangement are separate units of accounting if (i) the delivered item has value to the customer on a standalone basis and (ii) if

Onconova Therapeutics, Inc.**Notes to Consolidated Financial Statements (Continued)****2. Summary of Significant Accounting Policies (Continued)**

the arrangement includes a general right of return relative to the delivered item, delivery or performance of the undelivered item is considered probable and substantially within the Company's control. The arrangement consideration that is fixed or determinable at the inception of the arrangement is allocated to the separate units of accounting based on their relative selling prices. The appropriate revenue recognition model is applied to each element and revenue is accordingly recognized as each element is delivered. Management exercises significant judgment in determining whether a deliverable is a separate unit of accounting.

In determining the separate units of accounting, the Company evaluates whether the license has standalone value to the collaborator based on consideration of the relevant facts and circumstances for each arrangement. Factors considered in this determination include the research and development capabilities of the collaborator and the availability of relevant research expertise in the marketplace. In addition, the Company considers whether or not (i) the collaborator could use the license for its intended purpose without the receipt of the remaining deliverables, (ii) the value of the license was dependent on the undelivered items and (iii) the collaborator or other vendors could provide the undelivered items.

Under a collaborative research and license agreement, a steering committee is typically responsible for overseeing the general working relationships, determining the protocols to be followed in the research and development performed and evaluating the results from the continued development of the product. The Company evaluates whether its participation in joint steering committees is a substantive obligation or whether the services are considered inconsequential or perfunctory. The factors the Company considers in determining if its participation in a joint steering committee is a substantive obligation include: (i) which party negotiated or requested the steering committee, (ii) how frequently the steering committee meets, (iii) whether or not there are any penalties or other recourse if the Company does not attend the steering committee meetings, (iv) which party has decision making authority on the steering committee and (v) whether or not the collaborator has the requisite experience and expertise associated with the research and development of the licensed intellectual property.

Whenever the Company determines that an element is delivered over a period of time, revenue is recognized using either a proportional performance model, if a pattern of performance can be determined or a straight-line model over the period of performance, which is typically the research and development term. Progress achieved under the Company's various clinical research organization contracts are typically used as the measure of performance when applying the proportional performance method. At the end of each reporting period, the Company reassesses its cumulative measure of performance and makes appropriate adjustments, if necessary. The Company recognizes revenue using the proportional performance model whenever the Company is able to make reasonably reliable estimates of the level of effort required to complete its performance obligations under an arrangement. Revenue recognized under the proportional performance model at each reporting period is determined by multiplying the total expected payments under the contract (excluding royalties and payments contingent upon achievement of milestones) by the ratio of the level of effort incurred to date to the estimated total level of effort required to complete the performance obligations under the arrangement. Revenue is limited to the lesser of the cumulative amount of payments received or the cumulative amount of revenue earned, as determined using the proportional performance model as of each reporting period. Alternatively, if the Company is not able to make reasonably reliable estimates of the level of effort required to complete its performance obligations under an arrangement, then

Onconova Therapeutics, Inc.

Notes to Consolidated Financial Statements (Continued)

2. Summary of Significant Accounting Policies (Continued)

revenue under the arrangement is recognized on a straight-line basis over the period expected to be required to complete the Company's performance obligations.

Incentive milestone payments may be triggered either by the results of the Company's research efforts or by events external to it, such as regulatory approval to market a product or attaining agreed-upon sales levels. Consideration that is contingent upon achievement of a milestone is recognized in its entirety as revenue in the period in which the milestone is achieved, but only if the consideration earned from the achievement of a milestone meets all the criteria for the milestone to be considered substantive at the inception of the arrangement. For a milestone to be considered substantive, the consideration earned by achieving the milestone must (i) be commensurate with either the Company's performance to achieve the milestone or the enhancement of the value of the item delivered as a result of a specific outcome resulting from the Company's performance to achieve the milestone, (ii) relate solely to past performance and (iii) be reasonable relative to all deliverables and payment terms in the collaboration agreement.

For events for which the occurrences are contingent solely upon the passage of time or are the result of performance by a third party, the contingent payments will be recognized as revenue when payments are earned, the amounts are fixed and determinable and collectability is reasonably assured.

Royalties are recorded as earned in accordance with the contract terms when third party sales can be reliably measured and collectability is reasonably assured.

The Company recognized revenue of \$4,176,000 and \$45,490,000 during the years ended December 31, 2013 and 2012, respectively, as a result of its license and collaboration agreement with Baxter. The Company recognized revenue of \$577,000, \$503,000 and \$227,000 during the years ended December 31, 2013, 2012 and 2011, respectively, as a result of its license and collaboration agreement with SymBio. The remaining revenue recognized during the years ended December 31, 2012 and 2011 of \$197,000 and \$1,260,000, respectively, pertained to research and development services provided by the Company under certain research grants. The Baxter and SymBio agreements are the only agreements that are being accounted for under ASU 2009-13. See Note 15, "License and Collaboration Agreements," for a further discussion of the agreements with Baxter and SymBio.

Research and Development Expenses

Research and development costs are charged to expense as incurred. These costs include, but are not limited to, license fees related to the acquisition of in-licensed products; employee-related expenses, including salaries, benefits and travel; expenses incurred under agreements with contract research organizations and investigative sites that conduct clinical trials and preclinical studies; the cost of acquiring, developing and manufacturing clinical trial materials; facilities, depreciation and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance and other supplies; and costs associated with preclinical activities and regulatory operations.

Costs for certain development activities, such as clinical trials, are recognized based on an evaluation of the progress to completion of specific tasks using data such as patient enrollment, clinical site activations, or information provided to the Company by its vendors with respect to their actual costs incurred. Payments for these activities are based on the terms of the individual arrangements, which may differ from the pattern of costs incurred, and are reflected in the consolidated financial statements as prepaid or accrued research and development expense, as the case may be.

Onconova Therapeutics, Inc.**Notes to Consolidated Financial Statements (Continued)****2. Summary of Significant Accounting Policies (Continued)****Comprehensive Loss**

Comprehensive loss is defined as the change in equity of a business enterprise during a period from transactions and other events and circumstances from non-owner sources.

Income Taxes

The Company accounts for income taxes under the asset and liability method. Deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax bases using enacted tax rates in effect for the year in which the differences are expected to affect taxable income. The deferred tax asset primarily includes net operating loss and tax credit carry forwards, accrued expenses not currently deductible and the cumulative temporary differences related to certain research and patent costs, which have been charged to expense in the accompanying statements of operations but have been recorded as assets for income tax purposes. The portion of any deferred tax asset for which it is more likely than not that a tax benefit will not be realized must then be offset by recording a valuation allowance. A full valuation allowance has been established against all of the deferred tax assets (see Note 8, "Income Taxes"), as it is more likely than not that these assets will not be realized given the Company's history of operating losses. The Company recognizes the tax benefit from an uncertain tax position only if it is more likely than not to be sustained upon examination based on the technical merits of the position. The amount for which an exposure exists is measured as the largest amount of benefit determined on a cumulative probability basis that the Company believes is more likely than not to be realized upon ultimate settlement of the position.

Preferred Stock

The Company accounted for the redemption premium and issuance costs on its Preferred Stock using the effective interest method, accreting such amounts to its Preferred Stock from the date of issuance to the earliest date of redemption. Immediately prior to the consummation of the IPO, all outstanding shares of Preferred Stock automatically converted into shares of Common Stock at the applicable conversion ratio then in effect. As a result of the conversion, as of July 30, 2013, the Company had no shares of Preferred Stock outstanding. See Note 10.

Stock Warrants

Freestanding warrants that are related to the purchase of Stock are classified as liabilities and recorded at fair value. The warrants are subject to re-measurement at each balance sheet date and any change in fair value is recognized as a component of change in fair value of warrant liability in the consolidated statements of operations. See Note 10. The warrants are classified as Level 3 liabilities (see Note 4 for a discussion of the fair value hierarchy).

Stock-Based Compensation Expense

The Company applies the provisions of FASB Accounting Standards Codification ("ASC") Topic 718, Compensation—Stock Compensation ("ASC 718"), which requires the measurement and recognition of compensation expense for all stock-based awards made to employees and non-employees, including employee stock options.

Onconova Therapeutics, Inc.**Notes to Consolidated Financial Statements (Continued)****2. Summary of Significant Accounting Policies (Continued)**

At certain times throughout the Company's history, the chairman of the Company's board of directors, who is also a significant stockholder of the Company (the "Significant Holder"), afforded option holders the opportunity for liquidity in transactions in which options were exercised and the shares of Common Stock issued in connection therewith were simultaneously purchased by the Significant Holder (each, a "Purchase Transaction"). (See Note 11) Because the Company had established a pattern of providing cash settlement alternatives for option holders, the Company accounted for its stock-based compensation awards as liability awards. The Company measured liability awards based on the award's intrinsic value on the grant date and then re-measured them at each reporting date until the date of settlement. Compensation expense was recognized on a straight-line basis over the requisite service period for each separately vesting portion of the award. Compensation expense for each period until settlement was based on the change in intrinsic value (or a portion of the change in intrinsic value, depending on the percentage of the requisite service that has been rendered at the reporting date). Changes in the intrinsic value of a liability that occur after the end of the requisite service period were considered compensation expense in the period in which the changes occur. On April 23, 2013, the Company distributed a notification letter to all equity award holders under the 2007 Plan advising them that Purchase Transactions would no longer occur, unless, at the time of a Purchase Transaction, the option holder has held the Common Stock issued upon exercise of options for a period of greater than six months prior to selling such Common Stock to the Significant Holder and that any such sale to the Significant Holder would be at the fair value of the Common Stock on the date of such sale. Based on these new criteria for Purchase Transactions, the Company remeasured options outstanding under the 2007 Plan as of April 23, 2013 to their intrinsic value and reclassified such options from liabilities to stockholders' deficit within the Company's consolidated balance sheets, which amounted to \$14,482,000. The remaining expense for these options is being recognized on a straight-line basis over the remaining requisite service period.

Share-based payment transactions with employees, including grants of employee stock options, are recognized as compensation expense over the requisite service period based on their estimated fair values. ASC 718 also requires significant judgment and the use of estimates, particularly surrounding Black-Scholes assumptions such as stock price volatility over the option term and expected option lives, as well as expected option forfeiture rates, to estimate the grant date fair value of equity-based compensation and requires the recognition of the fair value of stock compensation in the statement of operations.

Clinical Trial Expense Accruals

As part of the process of preparing its financial statements, the Company is required to estimate its expenses resulting from its obligations under contracts with vendors, clinical research organizations and consultants and under clinical site agreements in connection with conducting clinical trials. The financial terms of these contracts are subject to negotiations, which vary from contract to contract and may result in payment flows that do not match the periods over which materials or services are provided under such contracts. The Company's objective is to reflect the appropriate trial expenses in its financial statements by matching those expenses with the period in which services are performed and efforts are expended. The Company accounts for these expenses according to the progress of the trial as measured by patient progression and the timing of various aspects of the trial. The Company determines accrual estimates through financial models taking into account discussion with applicable personnel and outside service providers as to the progress or state of consummation of trials, or the

Onconova Therapeutics, Inc.**Notes to Consolidated Financial Statements (Continued)****2. Summary of Significant Accounting Policies (Continued)**

services completed. During the course of a clinical trial, the Company adjusts its clinical expense recognition if actual results differ from its estimates. The Company makes estimates of its accrued expenses as of each balance sheet date based on the facts and circumstances known to it at that time. The Company's clinical trial accruals are dependent upon the timely and accurate reporting of contract research organizations and other third-party vendors. Although the Company does not expect its estimates to be materially different from amounts actually incurred, its understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and may result in it reporting amounts that are too high or too low for any particular period. For the years ended December 31, 2013, 2012 and 2011, there were no material adjustments to the Company's prior period estimates of accrued expenses for clinical trials.

Collaboration Arrangements

A collaboration arrangement is defined as a contractual arrangement that has or may have significant financial milestones associated with success-based development, which include certain arrangements the Company has entered into regarding the research and development, manufacture and/or commercialization of products and product candidates. These collaborations generally provide for non-refundable, upfront license fees, research and development and commercial performance milestone payments, cost sharing and royalty payments. The collaboration agreements with third-parties are performed on a "best efforts" basis with no guarantee of either technological or commercial success. The Company evaluates whether an arrangement is a collaboration arrangement at its inception based on the facts and circumstances specific to the arrangement. The Company reevaluates whether an arrangement qualifies or continues to qualify as a collaboration arrangement whenever there is a change in the anticipated or actual ultimate commercial success of the endeavor. See Note 15, "License and Collaboration Agreements," for a discussion of the Company's current collaborations with Baxter and SymBio.

Basic and Diluted Net Loss Per Share of Common Stock

Basic net loss per share of common stock is computed by dividing net loss applicable to common stockholders by the weighted-average number of shares of Common Stock outstanding during the period, excluding the dilutive effects of Preferred Stock, warrants to purchase Preferred Stock and stock options. Diluted net loss per share of common stock is computed by dividing the net loss applicable to common stockholders by the sum of the weighted-average number of shares of Common Stock outstanding during the period plus the potential dilutive effects of Preferred Stock and warrants to purchase Preferred Stock, and stock options outstanding during the period calculated in accordance with the treasury stock method, but are excluded if their effect is anti-dilutive. Because the impact of these items is anti-dilutive during periods of net loss, there was no difference between basic and diluted net loss per share of Common Stock for the years ended December 31, 2013, 2012 and 2011.

Recent Accounting Pronouncements

In February 2013, the Financial Accounting Standards Board (the "FASB") amended its guidance to require an entity to present the effect of certain significant reclassifications out of accumulated other comprehensive income on the respective line items in net income. The new accounting guidance does not change the items that must be reported in other comprehensive income or when an item of other comprehensive income must be reclassified to net income. The guidance is effective prospectively for

Onconova Therapeutics, Inc.**Notes to Consolidated Financial Statements (Continued)****2. Summary of Significant Accounting Policies (Continued)**

fiscal years beginning after December 15, 2012. The Company adopted these new provisions for the quarter beginning January 1, 2013. As the guidance requires additional presentation only, there was no impact to the Company's consolidated financial position, results of operations or cash flows.

In July 2013, the FASB issued guidance clarifying that an unrecognized tax benefit, or a portion of an unrecognized tax benefit, should be presented in the financial statements as a reduction to a deferred tax asset for a net operating loss carryforward, a similar tax loss, or a tax credit carryforward if such settlement is required or expected in the event the uncertain tax benefit is disallowed. In situations where a net operating loss carryforward, a similar tax loss, or a tax credit carryforward is not available at the reporting date under the tax law of the applicable jurisdiction or the tax law of the jurisdiction does not require, and the entity does not intend to use, the deferred tax asset for such purpose, the unrecognized tax benefit should be presented in the financial statements as a liability and should not be netted with the deferred tax asset. The guidance is effective for fiscal years, and interim periods within those years, beginning after December 15, 2013. Early adoption is permitted. The guidance is to be applied prospectively to all unrecognized tax benefits that exist at the effective date. Retrospective application is permitted. The Company is currently evaluating the impact that adoption will have on the determination or reporting of its financial results.

3. Property and Equipment

Property and equipment and related accumulated depreciation are as follows:

	<u>December 31,</u>	
	<u>2013</u>	<u>2012</u>
Laboratory equipment	\$ 866,000	\$ 764,000
Software	92,000	91,000
Computer and office equipment	433,000	323,000
Leasehold improvements	1,011,000	633,000
	<u>2,402,000</u>	<u>1,811,000</u>
Less accumulated depreciation	(1,776,000)	(1,348,000)
Property and equipment, net	<u>\$ 626,000</u>	<u>\$ 463,000</u>

Depreciation and amortization expense was \$449,000, \$319,000 and \$316,000 for the years ended December 31, 2013, 2012 and 2011, respectively.

4. Fair Value Measurements

The Company applies various valuation approaches in determining the fair value of its financial assets and liabilities within a hierarchy that maximizes the use of observable inputs and minimizes the use of unobservable inputs by requiring that observable inputs be used when available. Observable inputs are inputs that market participants would use in pricing the asset or liability based on market data obtained from sources independent of the Company. Unobservable inputs are inputs that reflect the Company's assumptions about the inputs that market participants would use in pricing the asset or

Onconova Therapeutics, Inc.**Notes to Consolidated Financial Statements (Continued)****4. Fair Value Measurements (Continued)**

liability and are developed based on the best information available under the circumstances. The fair value hierarchy is broken down into three levels based on the source of inputs as follows:

Level 1—Valuations based on unadjusted quoted prices in active markets for identical assets or liabilities that the Company has the ability to access.

Level 2—Valuations based on quoted prices for similar assets or liabilities in active markets, quoted prices for identical or similar assets or liabilities in markets that are not active and models for which all significant inputs are observable, either directly or indirectly.

Level 3—Valuations based on inputs that are unobservable and significant to the overall fair value measurement.

The availability of observable inputs can vary among the various types of financial assets and liabilities. To the extent that the valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair value requires more judgment. In certain cases, the inputs used to measure fair value may fall into different levels of the fair value hierarchy. In such cases, for financial statement disclosure purposes, the level in the fair value hierarchy within which the fair value measurement is classified is based on the lowest level input that is significant to the overall fair value measurement.

The Company had no assets or liabilities classified as Level 1 or Level 2. The Series G Preferred Stock warrants (see Note 10) are classified as Level 3. The fair values of these instruments are determined using models based on market observable inputs and management judgment. There were no material re-measurements of fair value during the years ended December 31, 2013 and 2012 with respect to financial assets and liabilities, other than those assets and liabilities that are measured at fair value on a recurring basis.

The Company has classified the Series G Preferred Stock warrants as a liability and has re-measured the liability to estimated fair value at December 31, 2013 and 2012, using the Black-Scholes option pricing model using the following assumptions: contractual life according to the remaining terms of the warrants, no dividend yield, weighted average risk-free interest rate of 0.34% and 0.31% at December 31, 2013 and 2012, respectively, and weighted average volatility of 74.40% and 64.87% at December 31, 2013 and 2012, respectively

The following fair value hierarchy table presents information about the Company's financial assets and liabilities measured at fair value on a recurring basis as of December 31, 2013 and 2012.

	Fair Value Measurement as of December 31, 2013				Fair Value Measurement as of December 31, 2012			
	Level 1	Level 2	Level 3	Balance	Level 1	Level 2	Level 3	Balance
Warrant liability	\$ —	\$ —	\$ 20,000	\$ 20,000	\$ —	\$ —	\$ 62,000	\$ 62,000
Total	\$ —	\$ —	\$ 20,000	\$ 20,000	\$ —	\$ —	\$ 62,000	\$ 62,000

Onconova Therapeutics, Inc.

Notes to Consolidated Financial Statements (Continued)

4. Fair Value Measurements (Continued)

The following table presents a reconciliation of the Company's liabilities measured at fair value on a recurring basis using significant unobservable inputs (Level 3) for the years ended December 31, 2013 and 2012:

	<u>Warrant Liability</u>
Balance at December 31, 2011	\$ 1,064,000
Settlements of warrant liability awards	(635,000)
Expiration of warrant liability awards	(975,000)
Change in fair value upon re-measurement	608,000
Balance at December 31, 2012	<u>62,000</u>
Change in fair value upon re-measurement	(42,000)
Balance at December 31, 2013	<u>\$ 20,000</u>

The fair values of cash equivalents, marketable securities, accounts payable and accrued liabilities approximate their respective carrying values due to the short-term nature of these accounts.

There were no transfers between Level 1 and Level 2 in any of the periods reported.

5. Net Loss Per Share of Common Stock

The following table sets forth the computation of basic and diluted earnings per share for the years ended December 31, 2013, 2012 and 2011:

	<u>Year ended December 31,</u>		
	<u>2013</u>	<u>2012</u>	<u>2011</u>
Basic and diluted net loss per share of common stock:			
Net loss	\$ (62,543,000)	\$ (29,912,000)	\$ (26,294,000)
Accretion to redemption value of preferred stock	(2,320,000)	(3,953,000)	(4,020,000)
Net loss applicable to common stockholders	<u>\$ (64,863,000)</u>	<u>\$ (33,865,000)</u>	<u>\$ (30,314,000)</u>
Weighted average shares of common stock outstanding	<u>10,594,227</u>	<u>2,206,888</u>	<u>2,137,403</u>
Net loss per share of common stock—basic and diluted	<u>\$ (6.12)</u>	<u>\$ (15.35)</u>	<u>\$ (14.18)</u>

Onconova Therapeutics, Inc.**Notes to Consolidated Financial Statements (Continued)****5. Net Loss Per Share of Common Stock (Continued)**

The following potentially dilutive securities outstanding at December 31, 2013, 2012 and 2011 have been excluded from the computation of diluted weighted average shares outstanding, as they would be antidilutive:

	December 31,		
	2013	2012	2011
Preferred Stock	—	12,838,127	8,575,918
Warrants	4,597	4,597	426,539
Stock options	4,344,365	2,564,147	2,026,396
	<u>4,348,962</u>	<u>15,406,871</u>	<u>11,028,853</u>

6. Balance Sheet Detail

Prepaid expenses and other current assets are as follows:

	December 31,	
	2013	2012
Research and development	\$ 2,242,000	\$ 1,429,000
Manufacturing	1,051,000	—
Insurance	645,000	101,000
Other	449,000	195,000
	<u>\$ 4,387,000</u>	<u>\$ 1,725,000</u>

Accrued expenses and other current liabilities are as follows:

	December 31,	
	2013	2012
Research and development	\$ 4,625,000	\$ 3,521,000
Employee compensation	509,000	247,000
Taxes	302,000	—
Other	384,000	157,000
	<u>\$ 5,820,000</u>	<u>\$ 3,925,000</u>

7. Debt

In December 2011, the chairman of the Company's board of directors, who is also a significant stockholder of the Company, advanced \$620,000 to the Company to fund operations. Interest accrued at 10% per annum. The stockholder loan was exchanged for convertible promissory notes in April 2012 (see Note 9).

8. Income Taxes

The Company accounts for income taxes under FASB ASC 740 ("ASC 740"). Deferred income tax assets and liabilities are determined based upon differences between financial reporting and tax bases

Onconova Therapeutics, Inc.

Notes to Consolidated Financial Statements (Continued)

8. Income Taxes (Continued)

of assets and liabilities, which are measured using the enacted tax rates and laws that will be in effect when the differences are expected to reverse.

Income taxes have been based on the following income (loss) before income tax expense:

	Year Ended December 31,		
	2013	2012	2011
Domestic	\$ (62,154,000)	\$ (29,912,000)	\$ (26,294,000)
Foreign	33,000	—	—
Total	\$ (62,121,000)	\$ (29,912,000)	\$ (26,294,000)

The provision for income taxes consists of the following:

	Year Ended December 31,		
	2013	2012	2011
Current			
US Federal	\$ 140,000	\$ —	\$ —
State and Local	285,000	—	—
Foreign	10,000	—	—
Total Current	\$ 435,000	\$ —	\$ —
Deferred			
US Federal	\$ —	\$ —	\$ —
State and Local	—	—	—
Foreign	—	—	—
Total Deferred	\$ —	\$ —	\$ —
Total Expense (Benefit)	\$ 435,000	\$ —	\$ —

As of December 31, 2013, the Company had federal net operating loss ("NOL") carry forwards of \$119,165,000, state NOL carry forwards of \$99,165,000 and research and development tax credit carry forwards of \$35,085,000, which are available to reduce future taxable income. The federal NOL and tax credit carry forwards will begin to expire at various dates starting in 2022. The state NOL carry forwards will begin to expire at various dates starting in 2016. The NOL carry forwards are subject to review and possible adjustment by the Internal Revenue Service and state tax authorities. NOL and tax credit carry forwards may become subject to an annual limitation in the event of certain cumulative changes in the ownership interest of significant shareholders over a three-year period in excess of 50%, as defined under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, as well as similar state tax provisions. This could limit the amount of NOLs that the Company can utilize annually to offset future taxable income or tax liabilities. The amount of the annual limitation, if any, will be determined based on the value of the Company immediately prior to the ownership change. Subsequent ownership changes may further affect the limitation in future years.

The Company's reserves related to taxes are based on a determination of whether and how much of a tax benefit taken by the Company in its tax filings or positions is more likely than not to be realized. The Company recognized no material adjustment for unrecognized income tax benefits.

Onconova Therapeutics, Inc.

Notes to Consolidated Financial Statements (Continued)

8. Income Taxes (Continued)

Through December 31, 2013, the Company had no unrecognized tax benefits or related interest and penalties accrued.

The principal components of the Company's deferred tax assets are as follows:

	Year Ended December 31,	
	2013	2012
Deferred tax assets:		
Net operating loss carryovers	\$ 47,364,000	\$ 35,921,000
R&D tax credits	35,223,000	13,868,000
Non-qualified stock options	3,988,000	1,057,000
Deferred revenue	5,938,000	4,246,000
Charitable contributions	6,000	6,000
Accrued expenses	586,000	40,000
Fixed assets	199,000	159,000
Deferred tax assets	93,304,000	55,297,000
Less valuation allowance	(93,304,000)	(55,297,000)
Net deferred tax assets	\$ —	\$ —

ASC 740 requires a valuation allowance to reduce the deferred tax assets reported if, based on the weight of available evidence, it is more likely than not that some portion or all of the deferred tax assets will not be realized. After consideration of all the evidence, both positive and negative, the Company has recorded a full valuation allowance against its deferred tax assets at December 31, 2013 and 2012, respectively, because the Company's management has determined that it is more likely than not that these assets will not be fully realized. The Company experienced a net change in valuation allowance of \$38,007,000 and \$8,718,000 for the years ended December 31, 2013 and 2012, respectively.

A reconciliation of income tax (expense) benefit at the statutory federal income tax rate and income taxes as reflected in the financial statements is as follows:

	Year Ended December 31,		
	2013	2012	2011
Federal income tax expense at statutory rate	34.0%	34.0%	34.0%
Permanent items	(8.3)	(25.1)	(5.6)
State income tax, net of federal benefit	4.5	1.8	6.2
Tax credits	34.4	18.5	22.1
Provision to return	(3.8)	0.0	(5.1)
Change in valuation allowance	(61.2)	(29.1)	(51.6)
Other	(0.2)	(0.1)	0.0
Effective income tax rate	(0.6)%	0.0%	0.0%

Onconova Therapeutics, Inc.

Notes to Consolidated Financial Statements (Continued)

9. Convertible Promissory Notes

In March 2012, the Company offered to its stockholders the opportunity to participate in a \$30,000,000 private placement of convertible promissory notes (the "Convertible Debt Offering"). From April through July 2012, the Company had aggregate closings of the Convertible Debt Offering of \$26,444,000, including \$620,000 from the principal that remained outstanding on a stockholder loan at December 31, 2011.

The convertible promissory notes issued in the Convertible Debt Offering carried a 10% interest rate calculated on the basis of a 360-day year and were scheduled to mature on June 29, 2013. The convertible promissory notes provided the holder the right to convert any portion of the outstanding principal and interest in exchange for equity instruments upon the completion of an initial public offering that generated aggregate proceeds in excess of \$25,000,000 (the "IPO Scenario") or upon the completion of an equity offering that generated aggregate proceeds in excess of \$15,000,000 (the "Equity Scenario"). In the event of a conversion under the IPO Scenario, the holder would have received shares of Common Stock equal to the offering price that was initially offered to the public. In the event of a conversion under the Equity Scenario, the holder would have received equity instruments equivalent to those issued in the Equity Scenario at a conversion price equal to the lower of \$11.00 per share or the original issuance price of the underlying equity instrument. If the Company merged with another company while the convertible promissory notes were still outstanding, immediately after which the Company's stockholders owned less than 50% of the voting stock of the surviving company, then each convertible promissory note would have been required to be redeemed for an amount equal to two times the outstanding principal amount together with any unpaid and accrued interest.

In July 2012, the Company amended and restated its certificate of incorporation and designated Series I Preferred Stock. The Company and the holders of the convertible promissory notes amended the Equity Scenario provision of the notes to permit the holders to convert the convertible promissory notes into shares of Series I Preferred Stock at a conversion price of \$11.00 per share.

In connection with the amendment of the convertible promissory notes, the notes were also analyzed to determine the existence of a beneficial conversion feature. The Company concluded that an \$8,176,000 contingent beneficial conversion feature existed related to the Equity Scenario as of July 2012. The fair value of the Series I Preferred Stock used to calculate the value of the beneficial conversion feature was determined by management with the assistance of a third-party valuation firm.

On July 27, 2012, the holders of the convertible promissory notes exercised their right to convert the outstanding principal and interest into Series I Preferred Stock. Upon conversion, the holders received 2,443,328 shares of Series I Preferred Stock, and the Company recorded interest expense of \$8,176,000, which was equal to the amount of the unamortized contingent beneficial conversion feature.

Onconova Therapeutics, Inc.

Notes to Consolidated Financial Statements (Continued)

10. Preferred Stock and Stockholders' Equity (Deficit)

Capitalization

The following is the composition of share capital as of December 31, 2013 and 2012:

	<u>Authorized</u>		<u>Issued and Outstanding</u>	
	<u>December 31, 2013</u>	<u>December 31, 2012</u>	<u>December 31, 2013</u>	<u>December 31, 2012</u>
Shares of \$0.01 par value per share:				
Common stock	75,000,000	30,145,155	21,467,482	2,606,484
Shares of \$0.01 par value per share:				
Preferred Stock	5,000,000	—	—	—
<i>Redeemable Preferred Stock:</i>				
Series A Preferred Stock	—	400,000	—	107,000
Series B Preferred Stock	—	1,200,000	—	1,107,189
Series C Preferred Stock	—	1,200,000	—	1,069,946
Series D Preferred Stock	—	1,625,000	—	1,583,568
Series E Preferred Stock	—	1,650,000	—	1,633,082
Series F Preferred Stock	—	2,000,000	—	2,000,000
Series G Preferred Stock	—	2,700,000	—	1,934,359
Series H Preferred Stock	—	2,042,950	—	2,013,424
Series I Preferred Stock	—	2,700,000	—	2,433,328
Series J Preferred Stock	—	3,030,303	—	3,030,303
Total Redeemable Preferred Stock	—	18,548,253	—	16,912,199

In preparation for the IPO, the Company's board of directors and stockholders approved a one-for-1.333 reverse stock split of the Company's Common Stock. The reverse stock split became effective on July 17, 2013. All Common Stock share and per share amounts in the condensed consolidated financial statements and notes thereto have been retroactively adjusted for all periods presented to give effect to this reverse stock split, including reclassifying an amount equal to the reduction in par value of common stock to additional paid-in capital. The reverse stock split did not result in a retroactive adjustment of share amounts for the Preferred Stock.

In July 2012, the Company issued 2,433,328 shares of Series I Preferred Stock in exchange for the conversion of the convertible promissory notes and accrued interest in the amount of \$26,444,000 and \$323,000, respectively. The effective conversion price was \$11.00 per share. Additionally, in July 2012, the Company issued 3,030,303 shares of Series J Preferred Stock at \$16.50 per share for gross proceeds of \$50,000,000. Issuance costs associated with this offering were \$2,204,000.

Series A Preferred Stock was issued at \$5.00 per share; Series B Preferred Stock was issued at \$5.75 per share; Series C Preferred Stock was issued at \$3.56 per share; Series D Preferred Stock was issued at \$4.67 per share; Series E Preferred Stock was issued at \$9.76 per share; Series F Preferred Stock was issued at \$11.00 per share; Series G and Series H Preferred Stock were issued at \$9.79 per share; Series I Preferred Stock was issued at \$11.00 per share; and Series J Preferred Stock was issued at \$16.50 per share.

Onconova Therapeutics, Inc.

Notes to Consolidated Financial Statements (Continued)

10. Preferred Stock and Stockholders' Equity (Deficit) (Continued)

The following is the activity of the Preferred Stock for the year ended December 31, 2013 and 2012:

	December 31, 2012	Issuance of Preferred Stock	Exercise of warrants	Accretion of redemption premium and issuance costs on Preferred Stock	Conversion of Preferred Stock into Common Stock	December 31, 2013
Series A						
Shares	107,000	—	—	—	(107,000)	—
Amount	\$ 535,000	\$ —	\$ —	\$ —	\$ (535,000)	\$ —
Series B						
Shares	1,107,189	—	—	—	(1,107,189)	—
Amount	\$ 12,733,000	\$ —	\$ —	\$ —	\$ (12,733,000)	\$ —
Series C						
Shares	1,069,946	—	—	—	(1,069,946)	—
Amount	\$ 7,618,000	\$ —	\$ —	\$ —	\$ (7,618,000)	\$ —
Series D						
Shares	1,583,568	—	—	—	(1,583,568)	—
Amount	\$ 18,211,000	\$ —	\$ —	\$ —	\$ (18,211,000)	\$ —
Series E						
Shares	1,633,082	—	—	—	(1,633,082)	—
Amount	\$ 18,780,000	\$ —	\$ —	\$ —	\$ (18,780,000)	\$ —
Series F						
Shares	2,000,000	—	—	—	(2,000,000)	—
Amount	\$ 23,000,000	\$ —	\$ —	\$ —	\$ (23,000,000)	\$ —
Series G						
Shares	1,934,359	—	—	—	(1,934,359)	—
Amount	\$ 22,819,000	\$ —	\$ —	\$ —	\$ (22,819,000)	\$ —
Series H						
Shares	2,013,424	—	—	—	(2,013,424)	—
Amount	\$ 22,005,000	\$ —	\$ —	\$ 868,000	\$ (22,873,000)	\$ —
Series I						
Shares	2,433,328	—	—	—	(2,433,328)	—
Amount	\$ 26,933,000	\$ —	\$ —	\$ 226,000	\$ (27,159,000)	\$ —
Series J						
Shares	3,030,303	—	—	—	(3,030,303)	—
Amount	\$ 48,681,000	\$ —	\$ —	\$ 1,226,000	\$ (49,907,000)	\$ —
Total						
Shares	16,912,199	—	—	—	(16,912,199)	—
Amount	\$ 201,315,000	\$ —	\$ —	\$ 2,320,000	\$ (203,635,000)	\$ —

Onconova Therapeutics, Inc.

Notes to Consolidated Financial Statements (Continued)

10. Preferred Stock and Stockholders' Equity (Deficit) (Continued)

	January 1, 2012	Issuance of Preferred Stock	Exercise of warrants	Accretion of redemption premium and issuance costs on Preferred Stock	December 31, 2012
Series A					
Shares	107,000	—	—	—	107,000
Amount	\$ 535,000	\$ —	\$ —	\$ —	\$ 535,000
Series B					
Shares	1,107,189	—	—	—	1,107,189
Amount	\$ 12,733,000	\$ —	\$ —	\$ —	\$ 12,733,000
Series C					
Shares	1,069,946	—	—	—	1,069,946
Amount	\$ 7,618,000	\$ —	\$ —	\$ —	\$ 7,618,000
Series D					
Shares	1,583,568	—	—	—	1,583,568
Amount	\$ 18,211,000	\$ —	\$ —	\$ —	\$ 18,211,000
Series E					
Shares	1,633,082	—	—	—	1,633,082
Amount	\$ 18,780,000	\$ —	\$ —	\$ —	\$ 18,780,000
Series F					
Shares	2,000,000	—	—	—	2,000,000
Amount	\$ 23,000,000	\$ —	\$ —	\$ —	\$ 23,000,000
Series G					
Shares	1,712,960	—	221,399	—	1,934,359
Amount	\$ 18,574,000	\$ —	\$ 2,802,000	\$ 1,443,000	\$ 22,819,000
Series H					
Shares	2,013,424	—	—	—	2,013,424
Amount	\$ 20,546,000	\$ —	\$ —	\$ 1,459,000	\$ 22,005,000
Series I					
Shares	—	2,433,328	—	—	2,433,328
Amount	\$ —	\$ 26,767,000	\$ —	\$ 166,000	\$ 26,933,000
Series J					
Shares	—	3,030,303	—	—	3,030,303
Amount	\$ —	\$ 47,796,000	\$ —	\$ 885,000	\$ 48,681,000
Total					
Shares	11,227,169	5,463,631	221,399	—	16,912,199
Amount	\$ 119,997,000	\$ 74,563,000	\$ 2,802,000	\$ 3,953,000	\$ 201,315,000

Voting

Prior to the consummation of the IPO, each holder of outstanding shares of Preferred Stock had the right to one vote for each share of Common Stock into which such Preferred Stock could then be converted. The holders of shares of Preferred Stock had full voting rights and powers equal to the voting rights and powers of shares of Common Stock and were entitled to notice of any stockholders'

Onconova Therapeutics, Inc.**Notes to Consolidated Financial Statements (Continued)****10. Preferred Stock and Stockholders' Equity (Deficit) (Continued)**

meeting and voted together with the holders of Common Stock, with respect to any question upon which holders of shares of Common Stock have the right to vote, as a single class, including without limitation, actions to increase or decrease the aggregate number of authorized shares of Common Stock and/or Preferred Stock.

Dividends

Prior to the consummation of the IPO, the holders of each share of Series A, Series B, Series C, Series D, Series E, Series F, Series G, Series H, Series I and Series J Preferred Stock were entitled to receive dividends when, as, and if declared by the Company's board of directors in the following order of preference: (i) the Series D, Series E, Series F, Series G, Series H, Series I and Series J Preferred Stock, which ranked pari passu; (ii) the Series B and Series C Preferred Stock, which ranked pari passu; (iii) the Series A Preferred Stock; and then (iv) Common Stock.

Liquidation

Prior to the consummation of the IPO, the assets of the Company legally available for distribution to stockholders were distributable in the following order of priority: (i) the holders of the shares of Series D, Series E, Series F, Series G, Series H, Series I and Series J Preferred Stock, which ranked pari passu; (ii) the holders of the shares of Series B and Series C Preferred Stock, which ranked pari passu; (iii) the holders of the shares of Series A Preferred Stock; and (iv) the holders of the shares of Common Stock. Each series of Preferred Stock was entitled to receive an amount per share equal to the greater of (1) the original issuance price for such series, plus all declared but unpaid dividends thereon, or (2) the amount that the holders of such series would receive per share of Common Stock if all shares of such series of Preferred Stock were converted to Common Stock immediately prior to such liquidation. If, upon a deemed liquidation event, the assets of the Company were insufficient to make payment in full to all holders of a series of Preferred Stock, then such assets would be distributed among the holders of such series of Preferred Stock at the time outstanding ratably in proportion to the full amount to which they would otherwise be respectively entitled. The holders of Common Stock were entitled to receive, after the payment of the liquidation preference of all Preferred Stock outstanding, the remaining assets of the Company on a pro rata basis.

Conversion

Prior to the consummation of the IPO, each issued and outstanding share of Preferred Stock was convertible into Common Stock at the holder's option at any time after the date of issuance or automatically upon the occurrence of certain events as defined in the Company's ninth amended and restated certificate of incorporation, at a defined conversion rate. Prior to the consummation of the IPO, the number of shares of Common Stock into which one share of each series of Preferred Stock was convertible was as follows, after giving effect to the reverse stock split discussed in Note 18: the Series A Preferred Stock, 0.80; the Series B Preferred Stock, 0.85; the Series C Preferred Stock, 0.75; the Series D Preferred Stock, 0.75; the Series E Preferred Stock, 0.75; the Series F Preferred Stock, 0.77; the Series G Preferred Stock, 0.75; the Series H Preferred Stock, 0.75; the Series I Preferred Stock, 0.75; and the Series J Preferred Stock, 0.75.

The conversion price for each share of Preferred Stock was subject to adjustment upon the occurrence of certain events. The conversion price of each share of a series of Preferred Stock was

Onconova Therapeutics, Inc.

Notes to Consolidated Financial Statements (Continued)

10. Preferred Stock and Stockholders' Equity (Deficit) (Continued)

adjusted if the Company issued additional shares, subject to specified exceptions, at a price lower than the then current conversion price for such series, which is measured and recognized if the contingency occurs.

On July 30, 2013, immediately prior to the consummation of the IPO, all outstanding shares of Preferred Stock automatically converted into shares of Common Stock at the applicable conversion ratio then in effect. See Note 10.

Redemption

To the extent it was then lawfully able to do so, the Company was required at any time, upon written request of the holders of at least 66.67% of the then outstanding Series A, Series B and Series C Preferred Stock collectively, or upon written request of the holders of at least a majority of the then outstanding shares of Series D, Series E and Series F Preferred Stock collectively, in each case as determined on an as-converted to Common Stock basis, to redeem the requested number of outstanding shares of Series A Preferred Stock at \$5.00 per share, Series B Preferred Stock at \$11.50 per share, and Series C Preferred Stock at \$7.12 per share, and/or Series D, E and F Preferred Stock at \$11.50 per share, as the case may be.

In addition, to the extent it was lawfully able to do so, the Company was required at any time, upon written request of the holders of at least a majority of the then outstanding shares of Series G Preferred Stock, to redeem, from the holders requesting such redemption, the requested number of outstanding shares of Series G Preferred Stock at \$11.50 per share.

To the extent it would have been lawfully able to do so, the Company would have been required at any time on or after September 21, 2013, upon written request of the holders of at least a majority of the then outstanding shares of Series H Preferred Stock, to redeem, from the holders requesting such redemption, the requested number of outstanding shares of Series H Preferred Stock at \$11.50 per share.

To the extent it would have been lawfully able to do so, the Company would have been required at any time on or after July 25, 2015, upon written request of the holders of at least a majority of the then outstanding shares of Series I Preferred Stock, to redeem, from the holders requesting such redemption, the requested number of outstanding shares of Series I Preferred Stock at \$11.50 per share.

To the extent it would have been lawfully able to do so, the Company would have been required at any time on or after July 27, 2015, upon written request of the holders of at least a majority of the then outstanding shares of Series J Preferred Stock, to redeem, from the holders requesting such redemption, the requested number of outstanding shares of Series J Preferred Stock at \$18.00 per share.

If, upon any applicable redemption date, defined as 60 days after the Company receives the written request for redemption, the funds of the Company legally available for redemption of Preferred Stock would have been insufficient to redeem the total number of shares to be redeemed on that date, those funds that were legally available would have been used to redeem the maximum possible number of shares, ratably among the holders of such shares to be redeemed. All remaining shares not redeemed would have remained outstanding until such time as additional funds became legally available for redemption.

Onconova Therapeutics, Inc.**Notes to Consolidated Financial Statements (Continued)****10. Preferred Stock and Stockholders' Equity (Deficit) (Continued)**

If more than one series of Preferred Stock had been contemporaneously subject to redemption, the redemption rights of the Preferred Stock would have followed the liquidation order of priority. As of December 31, 2012, Preferred Stock with an aggregate redemption value of \$103,122,000 was currently redeemable.

Warrant Transactions

The Company issued 6,128 Series G Preferred Stock warrants in connection with a loan and security agreement. Additionally, the Company issued one Series G Preferred Stock warrant for every two shares of Series G Preferred Stock purchased in 2009 and 2010. The warrants were initially recorded at their fair value calculated using the Black-Scholes model, with the following weighted average assumptions: exercise price of \$9.79, share price of \$9.79, expected term of three years, risk-free rate of 1.52% and volatility of 85.46%. The warrants are classified as liabilities because they are exercisable for Preferred Stock, and the value of the warrants is adjusted to current fair value at each reporting period end. For the years ended December 31, 2013, 2012 and 2011, the Company recorded \$42,000, \$367,000 and \$1,287,000, respectively, in the consolidated statements of operations related to the change in the fair value of the outstanding warrants.

Immediately prior to the consummation of the IPO, the 6,128 Series G Preferred Stock warrants outstanding were automatically converted into 4,597 Common Stock warrants (after giving effect to the one-for-1.333 reverse stock split).

11. Stock-Based Compensation

In January 2008, the board of directors approved the 2007 Equity Compensation Plan (the "2007 Plan"), which amended, restated and renamed the Company's 1999 Stock Based Compensation Plan (the "1999 Plan"), which provided for the granting of incentive and nonqualified stock options and restricted stock to its employees, directors and consultants at the discretion of the board of directors. Under the 2007 Plan, the Company increased the number of shares reserved for issuance under the 2007 Plan such that the number of reserved shares is equal to 17% of the fully diluted shares calculated annually on December 10th.

Further, in July 2013, the Company's board of directors and stockholders approved, effective immediately prior to the listing of the Common Stock on the NASDAQ Global Market, the 2013 Equity Compensation Plan (the "2013 Plan"), which amended, restated and renamed the 2007 Plan. Under the 2013 Plan, the Company may grant incentive stock options, non-statutory stock options, stock appreciation rights, restricted stock, restricted stock units, deferred share awards, performance awards and other equity-based awards to employees, directors and consultants. The Company initially reserved 6,107,831 shares of Common Stock for issuance, subject to adjustment as set forth in the 2013 Plan, of which 676,236 shares of Common Stock will be available for future issuance.

	<u>2013</u>	<u>2012</u>
Shares of Common Stock reserved for issuance under the 2013 Plan	<u>6,107,831</u>	<u>4,107,831</u>

Stock options may be granted with exercise prices of not less than the estimated fair value of the Common Stock on the date of grant and generally vest over a period of up to four years. Stock options

Onconova Therapeutics, Inc.

Notes to Consolidated Financial Statements (Continued)

11. Stock-Based Compensation (Continued)

granted under the 2013 Plan generally expire no later than ten years from the date of grant. A summary of stock option activity for the years ended December 31, 2013 and 2012 is as follows:

	Number of Options	Weighted- Average Exercise Price	Weighted- Average Remaining Contractual Term (in years)
Outstanding at December 31, 2010	1,823,266	\$ 4.41	6.09
Granted	344,479	6.13	
Exercised	(99,896)	1.53	
Forfeited	(41,453)	5.43	
Outstanding at December 31, 2011	2,026,396	\$ 4.81	6.39
Granted	977,807	12.51	
Exercised	(438,556)	2.90	
Forfeited	(1,500)	1.53	
Outstanding at December 31, 2012	2,564,147	8.10	7.52
Granted	1,967,940	14.60	
Exercised	(81,204)	1.93	
Forfeited	(106,518)	12.23	
Outstanding at December 31, 2013	4,344,365	\$ 11.05	7.91
Vested or expected to vest at December 31, 2013	4,344,365	\$ 11.05	7.91
Exercisable at December 31, 2013	2,347,744	\$ 8.72	6.63

At December 31, 2013 and 2012, the aggregate intrinsic value of the option liability recorded was \$0 and \$11,967,000, respectively. During the years ended December 31, 2013 and 2012, the Company granted 1,967,940 and 977,807 options at an intrinsic value of \$0 at the grant date.

At certain times throughout the Company's history, the Significant Holder has afforded option holders the opportunity for liquidity in transactions in which options were exercised and the shares of Common Stock issued in connection therewith were simultaneously purchased by the Significant Holder. Because the Company has established a pattern of providing cash settlement alternatives for option holders, the Company has accounted for its stock-based compensation awards as liability awards, the fair value of which is then re-measured at each balance sheet date. Upon the exercise of stock options from January 1, 2013 through April 23, 2013, stock option liabilities of \$38,000 were reclassified to stockholders' deficit.

On April 23, 2013, the Company distributed a notification letter to all equity award holders under the 2007 Plan advising them that Purchase Transactions would no longer occur, unless, at the time of a Purchase Transaction, the option holder has held the Common Stock issued upon exercise of options for a period of greater than six months prior to selling such Common Stock to the Significant Holder and that any such sale to the Significant Holder would be at the fair value of the Common Stock on the date of such sale. Based on these new criteria for Purchase Transactions, the Company remeasured options outstanding under the 2007 Plan as of April 23, 2013 to their intrinsic value and reclassified such options from liabilities to stockholders' deficit within the Company's consolidated balance sheets, which amounted to \$14,482,000.

Onconova Therapeutics, Inc.**Notes to Consolidated Financial Statements (Continued)****11. Stock-Based Compensation (Continued)**

A roll forward of the stock option liability balance for the years ended December 31, 2013 and 2012 is as follows:

Balance at December 31, 2011	\$ 2,648,000
Change in intrinsic value upon re-measurement	13,844,000
Settlements of option liability awards	(4,525,000)
Balance at December 31, 2012	11,967,000
Change in intrinsic value upon re-measurement	2,553,000
Settlements of option liability awards	(38,000)
Reclassification to stockholders' equity (deficit)	(14,482,000)
Balance at December 31, 2013	\$ —

The Company accounts for all stock-based payments made after April 23, 2013 to employees and directors using an option pricing model for estimating fair value. Accordingly, stock-based compensation expense is measured based on the estimated fair value of the awards on the date of grant, net of forfeitures. Compensation expense is recognized for the portion that is ultimately expected to vest over the period during which the recipient renders the required services to the Company using the straight-line single option method. In accordance with authoritative guidance, the fair value of non-employee stock based awards is re-measured as the awards vest, and the resulting increase in fair value, if any, is recognized as expense in the period the related services are rendered.

The Company estimates the fair value of its share-based awards to employees and directors using the Black-Scholes option pricing model. The Black-Scholes model requires the input of highly complex and subjective assumptions, including (a) the expected stock price volatility, (b) the calculation of the expected term of the award, (c) the risk free interest rate and (d) expected dividends. Due to the Company's limited operating history and a lack of company specific historical and implied volatility data, the Company has based its estimate of expected volatility on the historical volatility of a group of similar companies that are publicly traded. When selecting these public companies on which it has based its expected stock price volatility, the Company selected companies with comparable characteristics to it, including enterprise value, risk profiles, position within the industry, and with historical share price information sufficient to meet the expected life of the stock-based awards. The historical volatility data was computed using the daily closing prices for the selected companies' shares during the equivalent period of the calculated expected term of the stock-based awards. Due to its lack of sufficient historical data, the Company will continue to apply this process until a sufficient amount of historical information regarding the volatility of its own stock price becomes available. The Company has estimated the expected life of its employee stock options using the "simplified" method, whereby, the expected life equals the arithmetic average of the vesting term and the original contractual term of the option. The risk-free interest rates for periods within the expected life of the option are based on the U.S. Treasury yield curve in effect during the period the options were granted. The Company has never paid, and does not expect to pay dividends in the foreseeable future.

Stock-based compensation expense includes stock options granted to employees and non-employees and has been reported in the Company's statements of operations and comprehensive loss in either research and development expenses or general and administrative expenses depending on the function

Onconova Therapeutics, Inc.**Notes to Consolidated Financial Statements (Continued)****11. Stock-Based Compensation (Continued)**

performed by the optionee. No net tax benefits related to the stock-based compensation costs have been recognized since the Company's inception.

During the period from April 23, 2013 through December 31, 2013, the Company granted options to purchase shares of its Common Stock as follows:

<u>Shares</u>	<u>Exercise Price</u>
310,540	\$ 13.28
4,800	\$ 14.75
763,000	\$ 15.00
17,500	\$ 21.79
57,500	\$ 28.81
500	\$ 27.71
16,000	\$ 14.67
32,500	\$ 14.68
765,600	\$ 13.48

The Company recorded \$4,733,000 of compensation expense related to these options. The fair value of these options was estimated using the Black-Scholes option pricing model with the following weighted-average assumptions: no dividend yield, volatility of 77.00%, maximum contractual life of 5.87 years, and a risk-free interest rate of 1.73%.

The weighted-average grant-date fair value of stock options granted during the period from April 23, 2013 through December 31, 2013 and the years ended December 31, 2012 and 2011 was \$9.72, \$0.00, and \$0.00, respectively. The fair value of shares vested during the years ended December 31, 2013, 2012, and 2011 was \$9,875,000, \$5,554,000, and \$1,712,000, respectively.

The Company recognized stock-based compensation expense as follows for the years ended December 31, 2013 and 2012:

	<u>Year ended December 31,</u>		
	<u>2013</u>	<u>2012</u>	<u>2011</u>
General and administrative	\$ 4,845,000	\$ 7,199,000	\$ 3,000
Research and development	3,170,000	6,645,000	3,000
	\$ 8,015,000	\$ 13,844,000	\$ 6,000

As of December 31, 2013, there was \$1,875,000 of unrecognized compensation expense related to the unvested liability awards, which is expected to be recognized over a weighted-average period of approximately 2.32 years.

As of December 31, 2013, there was \$3,597,000 of unrecognized compensation expense related to the unvested stock options issued from April 23, 2013 through December 31, 2013, which is expected to be recognized over a weighted-average period of approximately 3.65 years. The Company accounts for stock options issued to nonemployees using a fair value approach.

Onconova Therapeutics, Inc.**Notes to Consolidated Financial Statements (Continued)****11. Stock-Based Compensation (Continued)**

Information with respect to stock options outstanding and exercisable at December 31, 2013 is as follows:

Exercise Price	Shares	Weighted Average Remaining Contractual Life (years)	Exercisable
\$1.33 - \$2.67	154,117	1.41	154,117
\$5.76 - 6.00	801,208	4.92	792,730
\$6.13 - \$7.53	639,888	6.41	553,790
\$13.28 - \$13.48	1,870,852	9.97	418,644
\$14.67 - \$15.00	805,300	9.59	421,244
\$21.79 - \$28.81	73,000	9.72	7,219
	<u>4,344,365</u>	<u>7.91</u>	<u>2,347,744</u>

The aggregate intrinsic value is calculated as the difference between the exercise price of the underlying awards and the quoted price of the Company's common stock for those awards that have an exercise price currently below the closing price. The intrinsic value of options exercised during the years ended December 31, 2013, 2012, and 2011 was \$1,076,000, \$4,510,000, and \$459,000, respectively. At December 31, 2013 the aggregate intrinsic value of options outstanding and vested options were \$10,212,000 and \$9,742,000, respectively.

12. Employee Benefit Plan

In October 2007, the Company established a 401(k) Retirement Savings Plan. Employees are eligible to participate in the plan as soon as they join the Company if they are at least 21 years of age and work a minimum of 1,000 hours per year. The Company matches \$0.50 (\$0.60 beginning January 1, 2014) for every dollar of the first 6% of payroll that employees invest, up to the legal limit. Employer contributions vest over four years at the rate of 25% per year. For the years ended December 31, 2013, 2012 and 2011, the Company contributed \$289,000, \$159,000 and \$131,000, respectively.

13. Commitments and Contingencies**Operating leases**

In November 2010, the Company entered into a lease for 3,117 square feet of office space in Pennington, New Jersey. The lease had an original term of two years, with an option for two additional years. For the first two years of the lease, the Company was obligated to pay \$4,400 per month, or \$53,000 annually, beginning when possession of the facility was taken on February 1, 2011. The Company was required to provide the landlord a \$125,000 letter of credit, the collateral for which is recorded as restricted cash on the consolidated balance sheets. This lease was renewed on February 1, 2012 with a 3.5% increase in the rent, to \$5,000 per month. On October 2, 2012, the Company leased an additional 2,130 square feet of office space for \$3,100 per month, or \$38,000 annually.

In January 2007, the Company entered into a lease for 8,100 square feet of office and lab space in Newtown, Pennsylvania, and in October 2009, the Company and the landlord amended the lease to add three additional one-year options to extend the lease term. The Company exercised the first option for the period from April 1, 2012 to March 31, 2013 and the second option for the period from April 1,

Onconova Therapeutics, Inc.**Notes to Consolidated Financial Statements (Continued)****13. Commitments and Contingencies (Continued)**

2013 to March 31, 2014 for rent of \$11,000 per month. In September 2012, the Company sub-leased an additional 1,356 square feet of office space for one year for \$1,600 per month, or \$19,000 annually.

Future minimum lease payments under these non-cancellable leases having terms in excess of one year as of December 31, 2013 are as follows:

	<u>December 31, 2013</u>
2014	\$ 245,000
2015	39,000
Total minimum lease payments	<u>\$ 284,000</u>

Rent expense was \$309,000, \$233,000 and \$186,000 for the years ended December 31, 2013, 2012 and 2011, respectively.

Employment agreements

The Company has entered into employment agreements with certain of its executives. The agreements provide for, among other things, salary, bonus and severance payments.

14. Research Agreements

The Company has entered into various licensing and right-to-sublicense agreements with educational institutions for the exclusive use of patents and patent applications, as well as any patents that may develop from research being conducted by such educational institutions in the field of anticancer therapy, genes and proteins. Results from this research have been licensed to the Company pursuant to these agreements. Under one of these agreements with Temple University ("Temple"), the Company is required to make annual maintenance payments to Temple and royalty payments based upon a percentage of sales generated from any products covered by the licensed patents, with minimum specified royalty payments. As no sales had been generated through December 31, 2013 under the licensed patents, the Company did not incur any royalty expenses for the years ended December 31, 2013, 2012 and 2011. In addition, the Company is required to pay Temple 25% of any sublicensing fees received by the Company. In September 2011, the Company made a payment to Temple in the amount of \$1,875,000 in connection with the collaboration agreement the Company executed in July 2011 with SymBio. Such payment was recorded in the consolidated statement of operations as research and development expenses. In 2012, the Company became obligated to pay Temple \$12,500,000 in connection with the collaboration agreement the Company executed in 2012 with Baxter. Such expense was recorded in the consolidated statement of operations as research and development expenses. As of December 31, 2012, \$1,405,000 of this amount was not yet paid and was included in accrued expenses and other current liabilities in the consolidated balance sheets. This amount was paid during 2013.

In May 2010, the Company signed a funding agreement with the Leukemia and Lymphoma Society ("LLS") to fund the development of rigosertib. Under this agreement, the Company was entitled to receive milestone payments of up to \$10,000,000 through 2013 in connection with clinical trials to be conducted. The aggregate milestone payment amount was subsequently reduced to \$8,000,000 pursuant to an amendment signed in January 2013, after which LLS was not obligated to fund any further amounts. In the event that the research is successful, the Company must proceed with commercialization of the licensed product or repay the amount funded. In addition, LLS is entitled to receive regulatory and commercial milestone payments and royalties from the Company based on the

Onconova Therapeutics, Inc.**Notes to Consolidated Financial Statements (Continued)****14. Research Agreements (Continued)**

Company's net sales of the licensed product, with the amount of such milestone payments and royalties not to exceed three times the amount funded (\$24,000,000). During the year ended December 31, 2012, in connection with the execution of the Baxter agreement (Note 15), the Company paid \$1,000,000 to LLS and recorded this amount in research and development expenses. This payment reduced the maximum milestone and royalty payment obligation under this agreement to \$23,000,000 at December 31, 2013 and 2012. As a result of the potential obligation to repay the funds under this arrangement, the milestone payments received through December 31, 2012 amounting to \$8,000,000 have been recorded as deferred revenue at December 31, 2013 and 2012 and will be recognized as revenue commencing with the resolution of the repayment contingency.

15. License and Collaboration Agreements**Baxter Agreement**

In September 2012, the Company entered into a development and license agreement with Baxter granting Baxter an exclusive, royalty-bearing license for the research, development, commercialization and manufacture (in specified instances) of rigosertib in all therapeutic indications in Europe (the "Baxter Territory"). In July 2012, Baxter purchased \$50.0 million of the Company's Series J convertible preferred stock, which converted to shares of Common Stock immediately prior to the consummation of the IPO. Baxter also invested \$5.0 million in the Company's initial public offering in July 2013.

Under the terms of the agreement, the Company was initially required to perform research and development to advance three initial rigosertib indications, rigosertib intravenous ("IV") in higher risk myelodysplastic syndrome ("MDS") patients, rigosertib IV in pancreatic cancer patients and rigosertib oral in lower risk MDS patients, through Phase 3, Phase 3 and Phase 2 clinical trials, respectively. In December 2013, a pre-planned interim futility and safety analysis of the pancreatic cancer trial was performed and the trial was discontinued. In February 2014, the Company announced top-line analysis of a Phase 3 trial of rigosertib IV in higher risk MDS patients. Although the results of this study showed numerical improvement in median overall survival in the rigosertib treated patients, the observed improvement of 2.4 months did not meet the required level of statistical significance. However, a statistically significant improvement in median overall survival was evident in the subset of patients who had progressed on or failed to respond to previous treatment with hypomethylating agents (HMAs).

If an additional Phase 3 clinical trial beyond the current Phase 3 clinical trial in process for rigosertib IV in higher risk MDS patients is required to obtain marketing approval in the Baxter Territory, the Company could require Baxter to fund a percentage of the costs of such additional trial up to a specified maximum. At the completion of the current Phase 2 trial for rigosertib oral in lower risk MDS patients and the review of the resulting data and findings, the Company and Baxter will decide whether or not to pursue further development of rigosertib for this indication. If the Company and Baxter mutually agree to progress the development of rigosertib oral in lower risk MDS patients, then certain milestone payments will be payable to the Company, and the Company will be required to use its commercially reasonable efforts to progress the development of rigosertib for this indication to a drug approval application in the Baxter Territory. The Company and Baxter will work together for potential future rigosertib indications, beyond the initial indications noted above. Generally, if Baxter chooses to participate in the development of additional indications, Baxter will be responsible for a percentage of all research and development costs and expenses and the Company could earn additional milestone payments. Baxter has full responsibility for all commercialization activities for the product in the Baxter Territory, at Baxter's sole cost and expense.

Onconova Therapeutics, Inc.**Notes to Consolidated Financial Statements (Continued)****15. License and Collaboration Agreements (Continued)**

The Company and Baxter have agreed to negotiate a supply agreement under terms satisfactory to both parties whereby the Company will supply Baxter with Baxter's required levels of product to support commercialization efforts in the Baxter Territory. Baxter also has the right to engage third parties for the manufacture and supply of its requirements for the licensed product.

Under the terms of the agreement, Baxter made an upfront payment of \$50,000,000. The Company is eligible to receive pre-commercial milestone payments of up to an aggregate of \$337,500,000 if specified development and regulatory milestones are achieved. The potential pre-commercial development milestone payments to the Company include the following:

- \$50,000,000 for achievement of the primary endpoint of a Phase 3 clinical trial for rigosertib IV in higher risk MDS patients (the "MDS IV indication") or mutual agreement of the Parties to file for any marketing approval in either the European Union as a whole or in all of the Big Five EU Countries;
- \$25,000,000 for the joint decision to proceed with the development of rigosertib for lower risk MDS; and
- \$25,000,000 for each drug approval application filed for indications specified in the arrangement with Baxter.

The Company may also receive up to \$212,500,000 in milestone payments for regulatory approvals of the rigosertib MDS indications specified in the arrangement with Baxter, each of which may be up to and in excess of \$100,000,000. The Company is also potentially eligible to receive an additional \$20,000,000 pre-commercial milestone payment related to the timing of regulatory approval of the MDS IV indication in Europe. In addition to these pre-commercial milestones, the Company is eligible to receive up to an aggregate of \$250,000,000 in milestone payments based on Baxter's achievement of pre-specified threshold levels of annual net sales of rigosertib. The Company will also be entitled to receive royalties at percentage rates ranging from the low-teens to the low-twenties on net sales of rigosertib by Baxter in the Baxter Territory.

The agreement with Baxter will remain in effect until the expiration of all applicable royalty terms and satisfaction of all payment obligations in each licensed country, unless terminated earlier due to the uncured material breach or bankruptcy of a party, force majeure, or in the event of a specified commercial failure. The Company may terminate the agreement in the event that Baxter brings a challenge against it in relation to the licensed patents. Baxter may terminate the agreement without cause commencing after a specified period of time from the execution of the agreement.

The Company determined that the deliverables under the Baxter agreement include the exclusive, royalty-bearing, sublicensable license to rigosertib and the research and development services to be performed by the Company. The Company concluded that the license had standalone value to Baxter and was separable from the research and development services because the license is sublicensable, there are no restrictions as to Baxter's use of the license and Baxter has significant research capabilities in this field.

In determining the separate units of accounting, the Company considered applicable accounting guidance and noted that in an arrangement with multiple deliverables, the delivered item or items shall be considered a separate unit of accounting if the delivered item or items have value to the customer on a stand-alone basis. The item or items have value on a stand-alone basis if they are sold separately

Onconova Therapeutics, Inc.**Notes to Consolidated Financial Statements (Continued)****15. License and Collaboration Agreements (Continued)**

by any vendor or the customer could resell the delivered item(s) on a stand-alone basis. In the context of a customer's ability to resell the delivered item(s), this criterion does not require the existence of an observable market for the deliverable(s).

The Baxter agreement allows Baxter to sublicense rigosertib and its ability to sublicense is not contingent on the approval or right of first refusal by the Company. The Company determined that Baxter's ability to sublicense the intellectual property to others demonstrates that the license has stand-alone value. In addition, at the time of entering into the Baxter agreement in September 2012, the rigosertib program was in a Phase 3 clinical trial for higher risk MDS, a Phase 3 clinical trial for pancreatic cancer and a Phase 2 trial for lower risk MDS. The protocols for the clinical trials had been written and provided to Baxter and a Special Protocol Assessment had already been granted to the Company by the FDA for higher risk MDS. These later stage clinical trials, where protocols have been prepared and trials are in process, can be completed more easily by entities other than the Company, as compared to earlier stage clinical trials. The remaining services to be performed by the Company are not proprietary and could be performed by other qualified parties. For example, the Company relies on clinical research organizations ("CROs") to complete the clinical trials, and Baxter could engage the same or similar CROs to complete the trials on its behalf. Although Baxter is not performing development activities related to rigosertib, Baxter possesses the internal expertise (or a vendor could be hired) to complete the efforts under the rigosertib programs without further assistance from the Company.

Baxter develops, manufactures and markets products that save and sustain the lives of people with hemophilia, immune disorders, infectious diseases, kidney disease, trauma, and other chronic and acute medical conditions. As a global, diversified healthcare company, Baxter applies a unique combination of expertise in medical devices, pharmaceuticals and biotechnology to create products that advance patient care worldwide. Baxter employs over 50,000 people, with significant revenues and expenditures for research and development. Baxter has expertise in completing clinical trials, assessing clinical trial results and preparing regulatory filings and has also developed and obtained regulatory and marketing approval in Europe for numerous products used to treat hematologic conditions. Baxter has expertise in rare hematologic conditions, and rigosertib is a natural complement to Baxter's existing treatments for patients with these conditions.

Baxter has the rights and full access to past and future intellectual information in order to obtain regulatory approval of rigosertib in Europe. In connection with the Baxter agreement, the Company licensed to Baxter all information and all patents controlled by the Company necessary for the development, manufacture, use and sale of rigosertib and all present and future formulations and dosages in all present and future therapeutic indications in the licensed territory.

Accordingly, given Baxter's ability to sublicense under the agreement and its ability internally or with outside help to conduct the ongoing development efforts, the Company concluded that the license has stand-alone value. In order to determine if the license can be treated as a separate unit of accounting, the Company also considered whether there is a general right of return associated with the license. The \$50,000,000 upfront payment received by the Company is non-refundable; therefore, there is no right of return for the license. As a result, the Company concluded that the license is a separate unit of accounting.

The Company was not able to establish VSOE or TPE for either the license or the research and development services and instead allocated the arrangement consideration between the license and

Onconova Therapeutics, Inc.**Notes to Consolidated Financial Statements (Continued)****15. License and Collaboration Agreements (Continued)**

research and development services based on their relative selling prices using BESP. Management developed the BESP of the license using a discounted cash flow model, taking into consideration assumptions including the development and commercialization timeline, discount rate and probability of success. Management utilized a third party valuation specialist to assist with the determination of BESP of the license. Management estimated the selling price of the research and development services using third party costs and a discounted cash flow model. The estimated selling prices utilized assumptions including internal estimates of research and development personnel needed to perform the research and development services; and estimates of expected cash outflows to third parties for services and supplies over the expected period that the services will be performed.

The key assumptions in these models included the following market conditions and entity-specific factors: (a) the specific rights provided under the license, (b) the stage of development of rigosertib and estimated remaining development and commercialization timelines, (c) the probability of successfully developing and commercializing rigosertib, (d) the market size including the associated sales figures which generate royalty revenue, (e) cost of goods sold, which was assumed to be a specified percentage of revenues based on estimated cost of goods sold of a typical oncology product, (f) sales and marketing costs, which were based on the costs required to field an oncology sales force and marketing group, including external costs required to promote an oncology product, (g) the expected product life of rigosertib assuming commercialization and (h) the competitive environment. The Company utilized a discount rate of 16%, representing the cost of capital derived from returns on equity for comparable companies.

Based on management's analyses, it was determined that the BESP of the license was \$120,000,000 and the BESP of the research and development services was \$20,600,000. As noted above, the Company received an up-front payment of \$50,000,000 under the Baxter agreement, which represents the allocable agreement consideration. Based on the respective BESP, this payment was allocated \$42,400,000 to the license and \$7,600,000 to the research and development services. Since the delivery of the license occurred upon the execution of the Baxter agreement and there was no general right of return, \$42,400,000 of the \$50,000,000 upfront payment was recognized upon the execution of the Baxter agreement. The portion allocated to research and development services is being recognized over the period of performance on a proportional performance basis through March 31, 2014. Management estimated the period of performance to be the period necessary for completion of the non-contingent obligations to perform research and development services required to advance the three formulations of rigosertib described above. For the years ended December 31, 2013 and 2012, the Company recognized \$4,176,000 and \$3,100,000, respectively, of research and development revenue under the Baxter agreement.

The Company and Baxter have agreed to establish a joint committee to facilitate the governance and oversight of the parties' activities under the agreements. Management considered whether participation on the joint committee may be a deliverable and determined that it was not a deliverable. Had management considered participation on the joint committee as a deliverable, it would not have had a material impact on the accounting for the arrangement based on the analysis of the estimated selling price of such participation.

As noted above, in July 2012, Baxter purchased Series J Preferred Stock. Because the Series J Preferred Stock was acquired within several months of the Baxter development and license agreement, management considered whether the Preferred Stock was issued at fair value and if not, whether the

Onconova Therapeutics, Inc.**Notes to Consolidated Financial Statements (Continued)****15. License and Collaboration Agreements (Continued)**

consideration received for the Preferred Stock (\$50,000,000) or for the collaboration and license agreement (\$50,000,000) should be allocated in the financial statements in a manner differently than the prices stated in the agreements. Management, with the assistance of an outside valuation specialist, determined that the price paid by Baxter for the Series J Preferred Stock approximated its fair value, and therefore the consideration received under the agreements was allocated in accordance with terms of the individual agreements.

SymBio Agreement

In July 2011, the Company entered into a license agreement with SymBio, as subsequently amended, granting SymBio an exclusive, royalty-bearing license for the development and commercialization of rigosertib in Japan and Korea. Under the SymBio license agreement, SymBio is obligated to use commercially reasonable efforts to develop and obtain market approval for rigosertib inside the licensed territory and the Company has similar obligations outside of the licensed territory. The Company has also entered into an agreement with SymBio providing for it to supply SymBio with development-stage product. Under the SymBio license agreement, the Company also agreed to supply commercial product to SymBio under specified terms that will be included in a commercial supply agreement to be negotiated prior to the first commercial sale of rigosertib. The supply of development-stage product and the supply of commercial product will be at the Company's cost plus a defined profit margin. Sales of development-stage product have been de minimis. The Company has additionally granted SymBio a right of first negotiation to license or obtain the rights to develop and commercialize compounds having a chemical structure similar to rigosertib in the licensed territory.

Under the terms of the SymBio license agreement, the Company received an upfront payment of \$7,500,000. The Company is eligible to receive milestone payments of up to an aggregate of \$22,000,000 from SymBio upon the achievement of specified development and regulatory milestones for specified indications. Of the regulatory milestones, \$5,000,000 is due upon receipt of marketing approval in the United States for rigosertib IV in higher risk MDS patients, \$3,000,000 is due upon receipt of marketing approval in Japan for rigosertib IV in higher risk MDS patients, \$5,000,000 is due upon receipt of marketing approval in the United States for rigosertib oral in lower risk MDS patients, \$5,000,000 is due upon receipt of marketing approval in Japan for rigosertib oral in lower risk MDS patients. Furthermore, upon receipt of marketing approval in the United States and Japan for an additional specified indication of rigosertib, which we are currently not pursuing, an aggregate of \$4,000,000 would be due. In addition to these pre-commercial milestones, the Company is eligible to receive tiered milestone payments based upon annual net sales of rigosertib by SymBio of up to an aggregate of \$30,000,000.

Further, under the terms of the SymBio license agreement, SymBio will make royalty payments to the Company at percentage rates ranging from the mid-teens to 20% based on net sales of rigosertib by SymBio.

Royalties will be payable under the SymBio agreement on a country-by-country basis in the licensed territory, until the later of the expiration of marketing exclusivity in those countries, a specified period of time after first commercial sale of rigosertib in such country, or the expiration of all valid claims of the licensed patents covering rigosertib or the manufacture or use of rigosertib in such country. If no valid claim exists covering the composition of matter of rigosertib or the use of or treatment with rigosertib in a particular country before the expiration of the royalty term, and specified

Onconova Therapeutics, Inc.**Notes to Consolidated Financial Statements (Continued)****15. License and Collaboration Agreements (Continued)**

competing products achieve a specified market share percentage in such country, SymBio's obligation to pay the Company royalties will continue at a reduced royalty rate until the end of the royalty term. In addition, the applicable royalties payable to the Company may be reduced if SymBio is required to pay royalties to third-parties for licenses to intellectual property rights necessary to develop, use, manufacture or commercialize rigosertib in the licensed territory. The license agreement with SymBio will remain in effect until the expiration of the royalty term. However, the SymBio license agreement may be terminated earlier due to the uncured material breach or bankruptcy of a party, or force majeure. If SymBio terminates the license agreement in these circumstances, its licenses to rigosertib will survive, subject to SymBio's milestone and royalty obligations, which SymBio may elect to defer and offset against any damages that may be determined to be due from the Company. In addition, the Company may terminate the license agreement in the event that SymBio brings a challenge against it in relation to the licensed patents, and SymBio may terminate the license agreement without cause by providing the Company with written notice within a specified period of time in advance of termination.

The Company determined that the deliverables under the SymBio agreement include the exclusive, royalty-bearing, sublicensable license to rigosertib, the research and development services to be provided by the Company and its obligation to serve on a joint committee. The Company concluded that the license did not have standalone value to SymBio and was not separable from the research and development services, because of the uncertainty of SymBio's ability to develop rigosertib in the SymBio territory on its own and the uncertainty of SymBio's ability to sublicense rigosertib and recover a substantial portion of the original upfront payment of \$7,500,000 paid by SymBio to the Company.

The supply of rigosertib for SymBio's commercial requirements is contingent upon the receipt of regulatory approvals to commercialize rigosertib in Japan and Korea. Because the Company's commercial supply obligation was contingent upon the receipt of future regulatory approvals, and there were no binding commitments or firm purchase orders pending for commercial supply at or near the execution of the agreement, the commercial supply obligation is deemed to be contingent and is not valued as a deliverable under the SymBio agreement. If SymBio orders the supplies from the Company, the Company expects the pricing for this supply to equal its third-party manufacturing cost plus a pre-negotiated percentage, which will not result in a significant incremental discount to market rates.

Due to the lack of standalone value for the license, research and development services, and joint committee obligation, the upfront payment is being recognized ratably using the straight line method through December 2027, the expected term of the agreement. For the years ended December 31, 2013, 2012 and 2011, the Company recognized revenues of \$455,000, \$455,000 and \$227,000, respectively, under this agreement. In addition, the Company recognized revenues of \$122,000 and \$48,000 for the years ended December 31, 2013 and 2012, respectively, related to the supply agreement with SymBio.

16. Preclinical Collaboration

In December 2012, we formed GBO, LLC, or GBO, an entity jointly-owned by both us and GVK Biosciences Private Limited, or GVK. The purpose of GBO is to collaborate on and develop two programs through filing of an investigational new drug application ("IND") and/or conducting proof of concept studies using the Company's technology platform.

During the year ended December 31, 2013, GVK made an initial capital contribution of \$500,000 in exchange for a 10% interest in GBO, and the Company made an initial capital contribution of a sub-license to all the intellectual property controlled by the Company related to the two specified

Onconova Therapeutics, Inc.**Notes to Consolidated Financial Statements (Continued)****16. Preclinical Collaboration (Continued)**

programs in exchange for a 90% interest. Under the terms of the joint venture agreement, GVK will make additional capital contributions (with a minimum of \$500,000) necessary to complete the major studies plus the exercise of the proof of concept study option leading to deliverables under the programs including an IND approval for one product and completion of IND studies for the other product. The GVK percentage interest in GBO accordingly may change from the initial 10% to up to 50%, depending on the amount of its total capital contributions. The Company evaluates its variable interests in GBO on a quarterly basis and has determined that it is the primary beneficiary.

For thirty days following the 15-month anniversary of the commencement of either of the two programs, the Company will have an option to (i) cancel the license and (ii) purchase all rights in and to that program. There are three of these buy-back scenarios depending on the stage of development of the underlying assets. GVK will have operational control of GBO and the Company will have strategic and scientific control.

17. Related-Party Transactions

The Company has entered into a research agreement, as subsequently amended, with the Mount Sinai School of Medicine ("Mount Sinai"), with which a member of its board of directors and a significant stockholder is affiliated. Mount Sinai is undertaking research on behalf of the Company on the terms set forth in the agreements. Mount Sinai, in connection with the Company, will prepare applications for patents generated from the research. Results from all projects will belong exclusively to Mount Sinai, but the Company will have an exclusive option to license any inventions. Payments to Mount Sinai for the years ended December 31, 2013, 2012 and 2011 were \$1,235,000, \$1,230,000 and \$554,000, respectively. At December 31, 2013 and 2012, the Company had no outstanding amounts payable to Mount Sinai.

The Company outsources the synthesis of some of its chemical compounds to vendors in the United States and in foreign countries. A supplier, of which a member of the Company's board of directors and a significant stockholder is an owner, produces one of these compounds under contract. The Company's aggregate payments for these services for the years ended December 31, 2013, 2012 and 2011 were \$0, \$157,000 and \$6,000, respectively. At December 31, 2013 and 2012, the Company owed this supplier \$0 and \$107,000, respectively, which is included in accounts payable on the consolidated balance sheets.

The Company purchases chemical compounds from corporations owned by a former member of its board of directors. The Company's aggregate payments to these suppliers for the years ended December 31, 2013, 2012 and 2011 were \$804,000, \$410,000 and \$970,000, respectively. At December 31, 2013 and 2012, the Company owed this supplier \$50,000 and \$0, respectively, which is included in accounts payable on the consolidated balance sheets. The Company also rents office space in Pennington, New Jersey from a corporation related to these suppliers and affiliated with the former member of the Company's board of directors.

The Company has entered into a consulting agreement, with a member of its board of directors, who is also a significant stockholder. The board member provides consulting services to the Company on the terms set forth in the agreement. Payments to this board member for the years ended December 31, 2013, 2012 and 2011 were \$182,000, \$165,000 and \$158,000, respectively.

Onconova Therapeutics, Inc.

Notes to Consolidated Financial Statements (Continued)

18. Initial Public Offering

On July 24, 2013, the Company's Registration Statement was declared effective by the SEC, and on July 25, 2013, the Company's Common Stock began trading on the NASDAQ Global Market under the symbol ONTX.

On July 30, 2013, immediately prior to the consummation of the IPO, all outstanding shares of Preferred Stock automatically converted into shares of Common Stock at the applicable conversion ratio then in effect. As a result of the conversion, as of September 30, 2013, the Company had no shares of Preferred Stock outstanding.

On July 30, 2013, the Company completed the IPO. The Company received net proceeds of \$79,811,000 from the IPO, net of underwriting discounts and commissions and other estimated offering expenses.

In preparation for the IPO, the Company's board of directors and stockholders approved a one-for-1.333 reverse stock split of the Company's Common Stock. The reverse stock split became effective on July 17, 2013. All Common Stock share and per share amounts in the consolidated financial statements and notes thereto have been retroactively adjusted for all periods presented to give effect to this reverse stock split, including reclassifying an amount equal to the reduction in par value of common stock to additional paid-in capital. The reverse stock split did not result in a retroactive adjustment of share amounts for the Preferred Stock. In addition, in July 2013, the Company's board of directors and stockholders approved an amendment of the Company's certificate of incorporation to, among other things, change the definition of a designated public offering to remove the per share price requirement and to set the threshold at gross proceeds to the Company of at least \$25.0 million.

Onconova Therapeutics, Inc.

Notes to Consolidated Financial Statements (Continued)

19. Quarterly Data (unaudited)

	2013			
	First Quarter	Second Quarter	Third Quarter	Fourth Quarter
Revenue	\$ 1,116,000	\$ 591,000	\$ 1,116,000	\$ 1,930,000
Operating expenses:				
General and administrative	3,346,000	3,117,000	5,927,000	4,403,000
Research and development	12,756,000	10,047,000	15,293,000	12,086,000
Total operating expenses	16,102,000	13,164,000	21,220,000	16,489,000
Loss from operations	(14,986,000)	(12,573,000)	(20,104,000)	(14,559,000)
Change in fair value of warrant liability	14,000	(2,000)	(31,000)	61,000
Interest expense	—	(2,000)	(1,000)	(1,000)
Other income, net	127,000	15,000	47,000	(126,000)
Net loss before income taxes	(14,845,000)	(12,562,000)	(20,089,000)	(14,625,000)
Income taxes	—	—	432,000	3,000
Net loss	(14,845,000)	(12,562,000)	(20,521,000)	(14,628,000)
Net loss attributable to non-controlling interest	—	—	—	13,000
Net loss attributable to Onconova Therapeutics, Inc	(14,845,000)	(12,562,000)	(20,521,000)	(14,615,000)
Accretion of redeemable convertible preferred stock	(1,019,000)	(1,032,000)	(269,000)	—
Net loss applicable to common stockholders	\$ (15,864,000)	\$ (13,594,000)	\$ (20,790,000)	\$ (14,615,000)
Net loss per share of common stock, basic and diluted*	\$ (6.08)	\$ (5.21)	\$ (1.34)	\$ (0.68)
Basic and diluted weighted average shares outstanding	2,607,406	2,609,495	15,480,416	21,419,208

Onconova Therapeutics, Inc.

Notes to Consolidated Financial Statements (Continued)

19. Quarterly Data (unaudited) (Continued)

	2012			
	First Quarter	Second Quarter	Third Quarter	Fourth Quarter
Revenue	\$ 198,000	\$ 220,000	\$ 42,803,000	\$ 2,969,000
Operating expenses:				
General and administrative	2,460,000	1,627,000	8,922,000	2,698,000
Research and development	8,448,000	6,776,000	26,962,000	10,576,000
Total operating expenses	10,908,000	8,403,000	35,884,000	13,274,000
Loss from operations	(10,710,000)	(8,183,000)	6,919,000	(10,305,000)
Change in fair value of warrant liability	(609,000)	999,000	(25,000)	2,000
Change in fair value option liability	—	—	—	—
Interest expense	(21,000)	(230,000)	(8,357,000)	—
Other income, net	541,000	(2,000)	28,000	41,000
Net loss before income taxes	(10,799,000)	(7,416,000)	(1,435,000)	(10,262,000)
Income taxes	—	—	—	—
Net loss	(10,799,000)	(7,416,000)	(1,435,000)	(10,262,000)
Net loss attributable to non-controlling interest	—	—	—	—
Net loss attributable to Onconova Therapeutics, Inc	(10,799,000)	(7,416,000)	(1,435,000)	(10,262,000)
Accretion of redeemable convertible preferred stock	(1,231,000)	(927,000)	(785,000)	(1,010,000)
Net loss applicable to common stockholders	\$ (12,030,000)	\$ (8,343,000)	\$ (2,220,000)	\$ (11,272,000)
Net loss per share of common stock, basic and diluted*	\$ (5.53)	\$ (3.84)	\$ (1.02)	\$ (4.89)
Basic and diluted weighted average shares outstanding	2,173,553	2,173,549	2,174,392	2,305,315

* Earnings Per Share (EPS) in each quarter is computed using the weighted-average number of shares outstanding during that quarter while EPS for the full year is computed using the weighted-average number of shares outstanding during the year. Thus, the sum of the four quarters' EPS does not equal the full-year EPS.

<u>Subsidiary</u>	<u>Jurisdiction of Incorporation</u>
Onconova Europe GmbH	Germany
GBO, LLC	Delaware

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[Exhibit 21.1](#)

Consent of Independent Registered Public Accounting Firm

We consent to the incorporation by reference in the Registration Statements (Form S-8 No. 333-191161 and Form S-8 No. 333-194228) pertaining to the Onconova Therapeutics, Inc. 2013 Equity Compensation Plan, of our report dated March 20, 2014, with respect to the financial statements of Onconova Therapeutics, Inc. included in this Annual Report (Form 10-K) for the year ended December 31, 2013.

/s/ Ernst & Young LLP

Philadelphia, Pennsylvania
March 20, 2014

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[EXHIBIT 23.1](#)

[Consent of Independent Registered Public Accounting Firm](#)

Consent of Independent Registered Public Accounting Firm

We consent to the incorporation by reference in the Registration Statement of Onconova Therapeutics, Inc. on Form S-8 (No. 333-191161 and No. 333-194228) of our report dated May 2, 2013, except for the third paragraph of Note 18, for which the date is July 17, 2013, on our audit of the financial statements for the year ended December 31, 2011, which report is included in this Annual Report on Form 10-K to be filed on or about March 20, 2014.

EISNERAMPER LLP

Iselin, New Jersey

March 20, 2014

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[EXHIBIT 23.2](#)

[Consent of Independent Registered Public Accounting Firm](#)

CERTIFICATIONS

I, Ramesh Kumar, certify that:

1. I have reviewed this Annual Report on Form 10-K of Onconova Therapeutics, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
 - (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
 - (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
 - (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
 - (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or other persons performing the equivalent functions):
 - (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

/s/ RAMESH KUMAR, PH.D.

Ramesh Kumar, Ph.D.
President and Chief Executive Officer
(Principal Executive Officer)

Dated: March 20, 2014

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[EXHIBIT 31.1](#)

[CERTIFICATIONS](#)

CERTIFICATIONS

I, Ajay Bansal, certify that:

1. I have reviewed this Annual Report on Form 10-K of Onconova Therapeutics, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
 - (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
 - (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
 - (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
 - (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or other persons performing the equivalent functions):
 - (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

/s/ AJAY BANSAL

Ajay Bansal
Chief Financial Officer
(Principal Financial Officer)

Dated: March 20, 2014

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[EXHIBIT 31.2](#)

[CERTIFICATIONS](#)

**CERTIFICATION
PURSUANT TO 18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with the Annual Report on Form 10-K of Onconova Therapeutics, Inc. (the "Company") for the year ended December 31, 2013 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Ramesh Kumar, Chief Executive Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, that:

- (1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and
- (2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

/s/ RAMESH KUMAR, PH.D.

Ramesh Kumar, Ph.D.
President and Chief Executive Officer
(Principal Executive Officer)

Dated: March 20, 2014

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[EXHIBIT 32.1](#)

[CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002](#)

**CERTIFICATION
PURSUANT TO 18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with the Annual Report on Form 10-K of Onconova Therapeutics, Inc. (the "Company") for the year ended December 31, 2013 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Ajay Bansal, Chief Financial Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, that:

- (1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and
- (2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

/s/ AJAY BANSAL

Ajay Bansal
Chief Financial Officer
(Principal Financial Officer)

Dated: March 20, 2014

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[EXHIBIT 32.2](#)

[CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002](#)