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March 02, 20 <b>FORM</b> Check thi if no long subject to Section 1 Form 4 or Form 5 obligation may cont <i>See</i> Instru 1(b).	<b>14</b> UNITEI	CMENT O ursuant to S 7(a) of the	Was F CHAN Section 14 Public Ut	Shington, GES IN I SECUR 6(a) of the	D.C. 20 BENEFI ITIES e Securit ling Com	549 CIA ies E ipany	L OWN xchange Act of	<b>COMMISSION</b> NERSHIP OF e Act of 1934, 1935 or Section 0	OMB Number: Expires: Estimated a burden hou response	
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Common Stock	02/28/2011			A	18	A	\$ 40.16	993	D	

Reminder: Report on a separate line for each class of securities beneficially owned directly or indirectly.

Persons who respond to the collection of information contained in this form are not required to respond unless the form displays a currently valid OMB control number.

 Table II - Derivative Securities Acquired, Disposed of, or Beneficially Owned

 (e.g., puts, calls, warrants, options, convertible securities)

1. Title of Derivative Security (Instr. 3)	2. Conversion or Exercise Price of Derivative Security	3. Transaction Date (Month/Day/Year)	3A. Deemed Execution Date, if any (Month/Day/Year)	4. Transacti Code (Instr. 8)	5. ionNumber of Derivative Securities Acquired (A) or Disposed of (D) (Instr. 3, 4, and 5)	;	ate	Secur	unt of rlying	8. Price of Derivative Security (Instr. 5)	9. Nu Deriv Secu Bene Owna Follo Repo Trans (Instr
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## **Reporting Owners**

<b>Reporting Owner Name / Address</b>	Relationships							
Reporting of the Funite / Funitess	Director	10% Owner	Officer	Othe				
Schnuck Todd R 1000 WALNUT ST., 7TH FLOOR KANSAS CITY, MO 64106	Х							
Signatures								
By: Jeffery D. Aberdeen For: Todd I Schnuck	₹.	03/0	1/2011					
**Signature of Reporting Person		D	ate					

## **Explanation of Responses:**

If the form is filed by more than one reporting person, see Instruction 4(b)(v).

\*\* Intentional misstatements or omissions of facts constitute Federal Criminal Violations. See 18 U.S.C. 1001 and 15 U.S.C. 78ff(a).

Note: File three copies of this Form, one of which must be manually signed. If space is insufficient, see Instruction 6 for procedure. Potential persons who are to respond to the collection of information contained in this form are not required to respond unless the form displays a currently valid OMB number. e='font-family:arial;margin-bottom:-11pt;'> the development of these product candidates may be adversely affected if we are unable to appropriately select patients for enrollment in our clinical trials;

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

we may not realize the full commercial potential of these product candidates that receive marketing approval if, among other reasons, we are unable to appropriately identify patients or types of tumors with the specific genetic alterations targeted by these product candidates. Even if our kinase inhibitor product candidates and any associated companion diagnostics are approved for marketing, the need for companion diagnostics may slow or limit adoption of our product candidates. Although we believe genetic testing is becoming more prevalent in the diagnosis and treatment of cancer, our product candidates may be perceived negatively compared to alternative treatments that do not require the use of companion diagnostics, either due to the additional cost of the companion diagnostic or the need to complete additional procedures to identify genetic markers prior to administering our product candidates.

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

# We may not be able to obtain an SPA prior to initiating Phase 3 clinical trials of mocetinostat. Even if obtained, an SPA would not guarantee any particular outcome from regulatory review.

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

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

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

In some cases there may be only one or few providers of such services, including clinical data management or manufacturing services. In addition, the cost of such services could increase significantly over time. We rely on third parties as mentioned above to enroll qualified patients and conduct, supervise and monitor our clinical trials. Our reliance on these third parties and collaborators for clinical development activities

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

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

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

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

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

inability to raise funding necessary to initiate or continue a trial;

delays in obtaining regulatory approval to commence a trial;

delays in reaching agreement with the FDA on final trial design;

imposition of a clinical hold following an inspection of our clinical trial operations or trial sites by the FDA or other regulatory authorities;

imposition of a clinical hold following an inspection of our clinical trial operations or trial sites by the FDA or other regulatory authorities;

delays in reaching agreement on acceptable terms with prospective CROs and clinical trial sites;

delays in obtaining required institutional review board approval at each site;

delays in recruiting suitable patients to participate in a trial;

delays in having subjects complete participation in a trial or return for post-treatment follow-up;

delays caused by subjects dropping out of a trial due to side effects or otherwise;

clinical sites dropping out of a trial to the detriment of enrollment;

time required to add new clinical sites; and

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delays by our contract manufacturers to produce and deliver a sufficient supply of clinical trial materials.

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

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

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

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

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

regulatory authorities may withdraw approvals of such product;

regulatory authorities may require additional warnings on the label;

we may be required to create a medication guide outlining the risks of such side effects for distribution to patients;

we could be sued and held liable for harm caused to patients; and

our reputation may suffer.

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

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

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

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Clinical trials may be delayed or suspended at any time by us or by the FDA or other similar regulatory authorities if it is determined at any time that patients may be or are being exposed to unacceptable health risks, including the risk of death, or if compounds are not manufactured under acceptable GMP conditions or with acceptable quality. Current regulations relating to regulatory approval may change or become more stringent. The agencies may also require additional trials be run in order to provide additional information regarding the safety, efficacy or equivalency of any compound for which we seek regulatory approval.

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

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

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

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

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



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adverse effect, the manufacturing of our products would also be adversely affected. Furthermore, key raw materials could become scarce or unavailable. There may be a limited number of third parties who can manufacture our products. We may not be able to meet specifications previously established for compounds during scale-up and manufacturing.

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

Contract manufacturers can encounter difficulties in achieving the scale-up, optimization, formulation, volume production of a compound as well as maintaining quality control with appropriate quality assurance. They may also experience shortages of qualified personnel. Contract manufacturers are required to undergo a satisfactory GMP inspection prior to regulatory approval and are obliged to operate in accordance with FDA, International Conference on Harmonisation of Technical Requirements for Registration of Pharmaceuticals for Human Use, or ICH, European and other nationally mandated GMP regulations and/or guidelines governing manufacturing processes, stability testing, record keeping and quality standards. A failure of these contract manufacturers to follow GMP and to document their adherence to such practices or failure of an inspection by a regulatory agency may lead to significant delays in the availability of material for clinical study, leading to delays in our trials.

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

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

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

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

demonstration of the clinical efficacy and safety of our products;

the prevalence and severity of any adverse side effects;

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

cost-effectiveness and availability of acceptable pricing;

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

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

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

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

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

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

Our and our collaborators' ability to commercialize our products successfully will depend, in part, on the extent to which coverage and adequate reimbursement for such products and related treatments will be available from governmental health payor programs at the federal and state levels, including Medicare and Medicaid, private health insurers, managed care plans and other organizations. No assurance can be given that third-party coverage and adequate reimbursement will be available that will allow us to maintain price levels sufficient for the realization of an appropriate return on our investment in product development.

Coverage and adequate reimbursement from governmental healthcare programs, such as Medicare and Medicaid, and commercial payors is critical to new product acceptance. Coverage decisions may depend upon clinical and economic standards that disfavor new drug products when more established or lower cost therapeutic alternatives are already available or subsequently become available. Even if we obtain coverage for our product candidates, the resulting reimbursement payment rates might not be adequate or may require co-payments that patients find unacceptably high. Patients are unlikely to use our product candidates unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of our product candidates.

In the United States, in Canada and in many other countries, pricing and/or profitability of some or all prescription pharmaceuticals and biopharmaceuticals are subject to varying degrees of government control. Healthcare reform and controls on healthcare spending may limit the price we charge for any products and the amounts thereof that we can sell. In particular, in the United States, the federal government and private insurers have changed and have considered ways to change, the manner in which healthcare services are provided. In March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or collectively, PPACA, became law in the United States. PPACA substantially changes the way healthcare is financed by both governmental and private insurers and significantly affects the healthcare industry. The provisions of PPACA of importance to our product candidates include the following:

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

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

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

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

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

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

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

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

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

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

In addition, other legislative changes have been proposed and adopted since PPACA was enacted. These changes include aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect on April 1, 2013. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, further reduced Medicare payments to several types of providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding, which could have a material adverse effect on our customers and accordingly, our financial operations.

We anticipate that PPACA, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and additional downward pressure on the reimbursement we may receive for any approved product. Moreover, payment methodologies may be subject to changes in healthcare legislation and regulatory initiatives. For example, the Middle Class Tax Relief and Job Creation Act of 2012 requires the Centers for Medicare & Medicaid Services, or CMS, to reduce the Medicare clinical laboratory fee schedule by 2% in 2013, which in turn will serve as a base for 2014 and subsequent years. CMS also recently proposed to re-examine payment amounts for tests reimbursed under the Medicare clinical laboratory fee schedule due to changes in technology and, in addition, proposed to bundle the Medicare payments for certain laboratory tests ordered while a patient received services in a hospital outpatient setting. The proposals would replace the current methodology for certain tests and, if adopted, the changes would begin to go into effect January 1, 2014 for some codes. Levels of reimbursement may be impacted by current and future legislation, regulation or reimbursement policies of third-party payors in a manner that may harm the demand and reimbursement available for our products, including our companion diagnostic, which in turn, could harm our future product pricing and sales. Any reduction in reimbursement from Medicare and other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize our products.

#### Explanation of Responses:

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

There are several hundred drugs in clinical development today in the area of oncology therapeutics. We have competitors both in the United States and internationally, including major multinational pharmaceutical companies, biotechnology companies and universities and other research institutions. In the oncology market, our major competitors include, but are not limited to: Amgen Inc.; ArQule Inc. and its partners Kyowa Hakko Kirin Pharma Inc. and Daiichi Sankyo Company Limited; Aveo Pharmaceuticals Inc.; Bristol-Myers Squibb Company; Exelixis Inc.; F. Hoffman-LaRoche Ltd.; GlaxoSmithKline PLC; Novartis AG; and Pfizer, among others.

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

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

Developments by others may render our products or technologies non-competitive or obsolete or we may not be able to keep pace with technological developments. Our competitors may have developed or may be developing technologies which may be the basis for competitive products. Some of these products may prove to be more effective and less costly than the products developed or being developed by us. Our competitors may obtain regulatory approval for their products more rapidly than we do which may change the standard of care in the indications we are targeting, rendering our technology or products non-competitive or obsolete. Others may develop treatments or cures superior to any therapy we are developing or will develop. Moreover, alternate, less toxic forms of medical treatment may be developed which may be competitive with our products.

Many of the organizations which could be considered to be our competitors have substantially more financial and technical resources, more extensive discovery research, preclinical research and development capabilities and greater manufacturing, marketing, distribution, production and human resources than we do. Many of our current or potential competitors have more experience than us in research, preclinical testing and clinical trials, drug commercialization, manufacturing and marketing, and in obtaining domestic and foreign regulatory approvals. In addition, failure, unacceptable toxicity, lack of sales or disappointing sales or other issues regarding competitors' products or processes could have a material adverse effect on our product candidates, including our clinical candidates or our lead compounds. Established pharmaceutical companies may invest heavily to accelerate discovery and development of novel compounds or to in-license novel compounds that could make our product candidates less competitive. In addition, any new product that competes with an approved product must demonstrate compelling advantages in efficacy, convenience, tolerability and safety in order to overcome price competition and brand recognition and to be commercially successful. Accordingly, our competitors may succeed in obtaining patent protection, receiving FDA, EMA or other regulatory approval or discovering, developing and commercializing medicines before we do, which would have a material adverse impact on our business.

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

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

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

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

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

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

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retainable. While we have been able to attract and retain skilled and experienced personnel and consultants in the past, no assurance can be given that we will be able to do so in the future.

# Our recently announced closure of our Canadian and New Jersey operations and related reduction in employees may disrupt our business, and we may not be able to adequately replace lost functionality through planned additional hiring at our San Diego facility or use of third-party service providers.

In connection with the Arrangement completed on June 28, 2013, we relocated our corporate headquarters from Montreal, Canada to San Diego, California. Since relocating to San Diego, we have maintained operations in our Canadian office. In addition to the ongoing operations in our Canadian office, we also maintain facilities in Princeton, New Jersey. On October 1, 2013, we announced our intention to close our New Jersey operations as of October 31, 2013 and to transition our Canadian operations to our San Diego offices over the next three to six months. In connection with these efforts, there will be a reduction in force of approximately 27 employees in our Montreal and Princeton offices, or approximately 75% of our workforce. We plan to partially offset this reduction in force by hiring additional personnel in our San Diego office and by engaging third-party service providers to perform certain functions. However, we may not be able to attract and retain the type and number of employees we desire in San Diego, or do so on our planned timeline. During this transition period, we may incur disruptions in our business, including from the loss of functionality we currently maintain in our Montreal and Princeton facilities. In addition, we may be unable to realize the efficiencies we are seeking by consolidating our operations in a single office in San Diego. If we are unable to realize such efficiencies or attract and retain qualified personnel in San Diego and effectively outsource certain other functions to third-party service providers, our operations in a single office in San Diego. If we are unable to realize such efficiencies and ability to execute our business plan would be adversely affected.

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

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

the federal Anti-Kickback Statute, which prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under federal and state healthcare programs, such as Medicare and Medicaid;

federal civil and criminal false claims laws and civil monetary penalty laws, including the federal False Claims Act, which impose criminal and civil penalties, including civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, including the Medicare and Medicaid programs, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government;

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

HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and their respective implementing regulations, which impose obligations on covered healthcare providers, health plans, and healthcare clearinghouses, as well as their business associates that create, receive, maintain or transmit individually identifiable health information for or on behalf of a covered entity, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;

the federal Open Payments program, which requires manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program, with specific exceptions, to report annually to CMS information related to "payments or other transfers of value" made to physicians, which is defined to include doctors, dentists, optometrists, podiatrists and chiropractors, and teaching hospitals and applicable manufacturers and applicable group purchasing organizations to report annually to CMS ownership and investment interests held by the physicians and their immediate family members, with data collection beginning on August 1, 2013, requirements for manufacturers to submit reports to CMS by March 31, 2014 and the 90th day of each subsequent calendar year, and disclosure of such information to be made by CMS on a publicly available website beginning in September 2014; and

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

Because of the breadth of these laws and the narrowness of available statutory and regulatory exceptions, it is possible that some of our business activities could be subject to challenge under one or more of such laws. In addition, recent healthcare reform legislation has strengthened these laws. For example, PPACA, among other things, amends the intent requirement of the federal Anti-Kickback Statute such that a person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it in order to have committed a violation. Moreover, PPACA provides that the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act. To the extent that any of our product candidates is ultimately sold in countries other than the United States, we may be subject to similar laws and regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, imprisonment, exclusion from participation in government healthcare programs, and the curtailment or restructuring of our operations, any of which could have a material adverse effect on our business. If any of the physicians or other healthcare providers or entities with whom we expect to do business, including any of our collaborators, is found not to be in compliance with applicable laws, it may be subject to criminal, civil or administrative sanctions, including exclusion from participation in government healthcare programs, which could also materially affect our business.

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

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

decreased demand for our product candidates;

injury to our reputation;

withdrawal of clinical trial participants;

initiation of investigations by regulators;

costs to defend the related litigation;

a diversion of management's time and our resources;

substantial monetary awards to trial participants or patients;

product recalls, withdrawals or labeling, marketing or promotional restrictions;

loss of revenue from product sales; and

the inability to commercialize any our product candidates, if approved.

We may not have or be able to obtain or maintain sufficient and affordable insurance coverage, and without sufficient coverage any claim brought against us could have a materially adverse effect on our business, financial condition or results of operations. We run clinical trials through investigators that could be negligent through no fault of our own and which could affect patients, cause potential liability claims against us and result in delayed or stopped clinical trials. We are required in many cases by contractual obligations to indemnify collaborators, partners, third-party contractors, clinical investigators and institutions. These indemnifications could result in a material impact due to product liability claims against us and/or these groups. We currently carry \$10 million in product liability insurance, which we believe is appropriate for our clinical trials. Although we maintain such insurance, any claim that may be brought against us could result in a court judgment or settlement in an amount that is not covered, in whole or in part, by our insurance or that is in excess of the limits of our insurance coverage. Our insurance policies also have various exclusions, and we may be subject to a product liability claim for which we have no coverage. We will have to pay any amounts awarded by a court or negotiated in a settlement that exceed our coverage limitations or that are not covered by our insurance, and we may not have, or be able to obtain, sufficient capital to pay such amounts.

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

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

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

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

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

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

Despite the implementation of security measures, any of the internal computer systems belonging to us, our collaborators or our third party service providers are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failure. Any system failure, accident or security breach that causes interruptions in our own, in collaborators' or in third party service vendors' operations could result in a material disruption of our drug discovery programs. To the extent that any disruption or security breach results in a loss or damage to our data or applications, or inappropriate disclosure of confidential or proprietary information, we may incur liability, our drug discovery programs may be adversely affected and the further development of our product candidates may be delayed. Furthermore, we may incur additional costs to remedy the damages caused by these disruptions or security breaches.

#### **Risks Relating to Our Intellectual Property**

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

Our success depends, in part, on our ability to secure and protect our patents, trade secrets, trademarks and other intellectual property rights and to operate without infringing on the proprietary rights of others or having third parties circumvent the rights that we own or license. We have filed and are actively pursuing patent applications in the United States, Canada, Japan, Europe and other major markets via the Patent Cooperation Treaty or directly in countries of interest. The patent positions of healthcare companies, universities and biopharmaceutical companies, including ours, are uncertain and involve complex questions



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of law and fact for which important legal issues may remain unresolved. Therefore, there is no assurance that our pending patent applications will result in the issuance of patents or that we will develop additional proprietary products which are patentable. Moreover, patents issued or to be issued to us may not provide us with any competitive advantage. Further, if the patent applications we hold or in-license with respect to our programs, product candidates and companion diagnostic fail to issue, if their breadth or strength of protection is threatened, or if they fail to provide meaningful exclusivity for our product candidates, it could dissuade companies from collaborating with us to develop product candidates, and threaten our ability to commercialize, future products.

Our patents may be challenged by third parties in patent litigation. In addition, it is possible that third parties with products that are very similar to ours will circumvent our patents by means of alternate designs or processes or file applications or be granted patents that would block or hurt our efforts. There are no assurances that our patent counsel, lawyers or advisors have given us correct advice or counsel. Opinions from such patent counsel or lawyers may not be correct or based on incomplete facts. We cannot be certain that we are the first to invent the inventions covered by pending patent applications and, if we are not, we may be subject to priority disputes. We may be required to disclaim part or all of the term of certain patents or all of the term of certain patent applications. There may be prior art of which we are not aware that may affect the validity or enforceability of a patent claim. There also may be prior art of which we are aware, but which we do not believe affects the validity or enforceability of a claim, which may, nonetheless, ultimately be found to affect the validity or enforceability of a claim. No assurance can be given that if challenged, our patents would be declared by a court to be valid or enforceable or that even if found valid and enforceable, a competitor's technology or product would be found by a court to infringe our patents. We may analyze patents or patent applications of our competitors that we believe are relevant to our activities, and consider that we are free to operate in relation to our product candidates, but our competitors may achieve issued claims, including in patents we consider to be unrelated, which block our efforts or may potentially result in our product candidates or our activities infringing such claims. The possibility exists that others will develop products which have the same effect as our products on an independent basis which do not infringe our patents or other intellectual property rights, or will design around the claims of patents that we have had issued that cover our products. The steps we have taken to protect our intellectual property may not prevent the misappropriation of our proprietary information and technologies, particularly in foreign countries where laws or law enforcement practices may not protect proprietary rights as fully as in Canada, the United States or Europe. Unauthorized disclosure of our proprietary information could also harm our competitive position. We could also inadvertently use our collaborators' data inappropriately which could lead to liability. We may file patent applications but have claims restricted or we may not be able to supply sufficient data to satisfy a patent office to support our claims and, as a result, may not obtain the original claims desired or we may receive restricted claims. Alternatively, it is possible that we may not receive any patent protection from an application.

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

Many of our collaboration agreements are complex and may call for licensing or cross-licensing of potentially blocking patents, know-how or intellectual property. Due to the potential overlap of data, know-how and intellectual property rights there can be no assurance that one of our collaborators will not

dispute our right to send data or know-how or other intellectual property rights to third parties and this may potentially lead to liability or termination of a program. There are no assurances that the actions of our collaborators would not lead to disputes or cause us to default with other collaborators. We cannot be certain that a collaborator will not challenge the validity of licensed patents.

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

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

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

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

Patent applications which may relate to or affect our business may have been filed by others. Such patent applications or patents resulting therefrom may conflict with our technologies, patents or patent applications and reducing the scope of our patent protection. Such events could cause us to stop or change the course of our research and development or modify our intellectual property strategies. We could also become involved in interference proceedings in connection with one or more of our patents or patent applications to determine priority of invention. There can be no guarantees that an interference proceeding would be successful or that such an outcome could be reversed on appeal. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Our defense of such interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees.



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

Third parties may assert that we are using their proprietary information without authorization. Third parties may also have or obtain patents and may claim that technologies licensed to or used by us infringe their patents. Because patent applications can take many years to issue, third parties may have currently pending patent applications which may later result in issued patents that our product candidates or companion diagnostic may infringe, or which such third parties claim are infringed by the use of our technologies. If any third-party patents are held by a court of competent jurisdiction to cover any aspect of our product candidates, including the formulation or method of use of such product candidate, the holders of any such patents may be able to block our ability to commercialize such product candidate unless we obtained a license under the applicable patents, or until such patents expire. In any such case, such a license may not be available on commercially reasonable terms or at all. In addition, any legal action that seeks damages or an injunction to stop us from carrying on our commercial activities relating to the affected technologies could subject us to monetary liability. Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources.

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

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

Third parties may infringe one or more of our issued patents or trademarks. We cannot predict if, when or where a third party may infringe one or more of our issued patents or trademarks. We may attempt to invalidate a competitor's patent. There is no assurance such action will ultimately be successful and, even if initially successful, it could be overturned upon appeal. There is no assurance that we would be successful in a court of law to prove that a third party is infringing one or more of our issued patents. Even if we are successful in proving in a court of law that a third party is infringing one or more of our issued patents. Even if we are successful in halting their infringing activities, for example, through a permanent injunction, or that we would be fully or even partially financially compensated for any harm to our business. We may be forced to enter into a license or other agreement with the infringing third party at terms less profitable or otherwise less commercially acceptable to us than if the license or agreement were negotiated under conditions between those of a willing licensee and a willing licensor. We may not become aware of a third party infringer within legal timeframes that would enable us to seek adequate compensation, or at all, thereby possibly losing the ability to be compensated for any harm to our business. Such a third-party infringer is difficult to locate, where we do not have issued patents and/or the patent laws may be more difficult to enforce. Some third-party infringers may be able to sustain the costs of complex patent infringement litigation more effectively than we can because they have substantially greater resources. Any inability to stop third-party infringement could result in loss in market share of some of our products or even lead to a delay, reduction

and/or inhibition of the development, manufacture or sale of certain products by us. There is no assurance that a product produced and sold by a third-party infringer would meet our or other regulatory standards or would be safe for use. Such third-party infringer products could irreparably harm the reputation of our products thereby resulting in substantial loss in market share and profits.

#### **Risks Related to Our Shares of Common Stock**

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

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

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

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

Based on the information available to us as of August 31, 2013, prior to this offering our stockholders and their affiliates who owned more than 5% of our outstanding common stock collectively owned approximately 81% of our outstanding common stock. Prior to this offering, Baker Brothers and Tavistock and their affiliates collectively owned approximately 40% of our outstanding common stock. In addition, in conjunction with certain financing transactions, we granted to Baker Brothers and Tavistock each the right to nominate a member of our Board of Directors and the right to appoint an observer on our Board of Directors. As a result, each of Baker Brothers and Tavistock has significant influence over matters submitted to our stockholders for approval, including the election and removal of directors and the approval of any merger, consolidation, or sale of all or substantially all of our assets. Furthermore, as a thinly traded stock, if Baker Brothers, Tavistock or any of other of our major stockholders determine to exit from the industry or from their holdings in us, for whatever reason, the impact on our share price could be detrimental over a prolonged period of time.



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

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

Pursuant to our 2013 Equity Incentive Plan, or the 2013 Plan, our management is authorized to grant stock options and other equity-based awards to our employees, directors and consultants. Any increase in the number of shares outstanding as a result of the exercise of outstanding options will cause our stockholders to experience additional dilution, which could cause our stock price to fall. Currently, we plan to register the increased number of shares available for issuance under the 2013 Plan each year.

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

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

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

We are a holding company with no material assets other than the stock of our wholly-owned subsidiary. Accordingly, all our operations are conducted by MethylGene Canada, our wholly-owned subsidiary (and its wholly-owned subsidiary, MethylGene US Inc.). MethylGene Canada has never declared or paid any cash dividends on its common shares, and we currently expect that the earnings and cash flow of MethylGene Canada will primarily be retained and used by it in its operations, including servicing any debt obligations it may have now or in the future. Accordingly, although we do not anticipate paying any dividends in the foreseeable future, our subsidiary may not be able to generate sufficient cash flow to distribute funds to us in order to allow us to pay future dividends on, or make any distributions with respect to our common stock. As a result, capital appreciation, if any, of our common stock would be our stockholders' sole source of gain on their investment in our common stock for the foreseeable future.

#### **Risks Related to This Offering**

#### Management will have broad discretion as to the use of the proceeds from this offering, and we may not use the proceeds effectively.

Our management will have broad discretion in the application of the net proceeds from this offering and could spend the proceeds in ways that do not improve our results of operations or enhance the value of our common stock. Our failure to apply these funds effectively could have a material adverse effect on our business, delay the development of our product candidates and cause the price of our common stock to decline.

# If you purchase the common stock sold in this offering, you will experience immediate and substantial dilution in your investment. You will experience further dilution if we issue additional equity securities in future fundraising transactions.

Since the price per share of our common stock being offered is substantially higher than the net tangible book value per share of our common stock, you will suffer substantial dilution with respect to the net tangible book value of the common stock you purchase in this offering. Based on the public offering price of \$17.50 per share and our net tangible book value as of June 30, 2013, if you purchase shares of common stock in this offering, you will suffer immediate and substantial dilution of \$13.02 per share with respect to the net tangible book value of the common stock. See "Dilution" for a more detailed discussion of the dilution you will incur if you purchase common stock in this offering.

In addition, we have a significant number of stock options and warrants outstanding. To the extent that outstanding stock options or warrants have been or may be exercised or other shares issued, investors purchasing our common stock in this offering may experience further dilution. In addition, after this offering we may choose to raise additional capital due to market conditions or strategic considerations even if at the time we believe we have sufficient funds for our current or future operating plans. To the extent that additional capital is raised through the sale of equity or convertible debt securities, the issuance of these securities could result in further dilution to our stockholders or result in downward pressure on the price of our common stock.

#### CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus contains "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933, as amended, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act. The forward-looking statements are contained principally in the sections entitled "Prospectus Summary," "Risk Factors," "Management's Discussion and Analysis of Financial Condition and Results of Operations" and "Business" in this prospectus. These statements relate to future events or to our future financial performance and involve known and unknown risks, uncertainties and other factors which may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements.

Forward-looking statements include, but are not limited to, statements about:

the initiation, cost, timing, progress and results of our research and development activities, preclinical studies and future clinical trials;

our ability to obtain and maintain regulatory approval for our product candidates, and any related restrictions, limitations, and/or warnings in the label of any approved product candidate;

our ability to obtain funding for our operations;

our plans to research, develop and commercialize our future product candidates;

our strategic partners' decisions relating to development and commercialization of product candidates;

our ability to attract collaborators with development, regulatory and commercialization expertise;

our ability to obtain and maintain intellectual property protection for our future product candidates;

the size and growth potential of the markets for our future product candidates, and our ability to serve those markets;

our ability to successfully commercialize our future product candidates;

the rate and degree of market acceptance of our future product candidates;

our ability to develop sales and marketing capabilities, whether alone or with potential future collaborators;

regulatory developments in the United States and foreign countries;

the performance of our third-party suppliers and manufacturers;

the success of competing therapies that are or become available;

the loss of key scientific or management personnel;

the expected impact of the closures of our Montreal and Princeton offices and the associated changes to our workforce;

our use of the proceeds from this offering; and

the accuracy of our estimates regarding cash, cash equivalents and marketable securities as of September 30, 2013, expenses, future revenue, capital requirements and need for additional financing.

In some cases, you can identify these statements by terms such as "anticipate," "believe," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "should," "will," "would" or the negative of those terms, and similar expressions. These forward-looking statements reflect our management's beliefs and views with respect to future events and are based on estimates and assumptions as of the date of this prospectus and are subject to risks and uncertainties. We discuss many of these risks in greater detail under the heading "Risk Factors." Moreover, we operate in a very competitive and rapidly changing environment. New risks emerge from time to time. It is not possible for our management to predict all risks, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any

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forward-looking statements we may make. Given these uncertainties, you should not place undue reliance on these forward-looking statements. For all forward-looking statements, we claim the protection of the safe harbor for forward-looking statements contained in the Private Securities Litigation Reform Act of 1995.

You should read this prospectus and the documents that we reference in this prospectus and have filed as exhibits to the registration statement, of which this prospectus is a part, completely and with the understanding that our actual future results may be materially different from what we expect. We qualify all of the forward-looking statements in this prospectus by these cautionary statements.

Except as required by law, we assume no obligation to update these forward-looking statements publicly, or to update the reasons actual results could differ materially from those anticipated in these forward-looking statements, even if new information becomes available in the future.

#### MARKET, INDUSTRY AND OTHER DATA

Unless otherwise indicated, information contained in this prospectus concerning our industry and the market in which we operate, including our market position, market opportunity and market size, is based on information from various sources, on assumptions that we have made based on such data and other similar sources and on our knowledge of the markets for our products. These data involve a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates.

While we believe the market position, market opportunity and market size information included in this prospectus is generally reliable, such information is inherently imprecise. In addition, projections, assumptions and estimates of our future performance and the future performance of the industry in which we operate is necessarily subject to a high degree of uncertainty and risk due to a variety of factors, including those described in the section entitled "Risk Factors" and elsewhere in this prospectus. These and other factors could cause results to differ materially from those expressed in the estimates made by the independent parties and by us.

#### **USE OF PROCEEDS**

We estimate that we will receive net proceeds of approximately \$53.0 million (or approximately \$61.0 million if the underwriters' option to purchase additional shares is exercised in full) from the sale of the shares of common stock offered by us in this offering, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us.

We intend to use the net proceeds of this offering:

to fund our planned Phase 1 dose expansion cohort trials with MGCD265 and a subsequent Phase 2 clinical trial for MGCD265;

to fund our planned Phase 1 dose expansion cohorts with MGCD516;

to fund our planned open-label, dose confirmation clinical trial with mocetinostat; and

for other research and development activities, working capital and general corporate purposes.

We may also use a portion of the net proceeds to in-license, acquire or invest in complementary businesses or products; however, we have no current commitments or obligations to do so. Based upon our current plans, we anticipate that the net proceeds from this offering, along with our existing cash and investment balances, will fund our operations through the end of 2015, including the completion of the planned clinical trials referenced above. We have based these estimates on assumptions that may prove to be wrong, and we could use our capital resources sooner than we currently expect. Pursuant to our current plans, we do not anticipate initiating Phase 3 trials with mocetinostat absent additional financing or the establishment of a collaboration for late-stage development. We will have broad discretion in the use of the net proceeds from this offering and could spend the proceeds in ways that do not improve our results of operations or enhance the value of our stock. Pending their use, we plan to invest the net proceeds from this offering in short- and intermediate-term, interest-bearing obligations, investment-grade instruments, certificates of deposit or direct or guaranteed obligations of the U.S. government.

#### PRICE RANGE OF OUR COMMON STOCK

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

On October 23, 2013, the last reported sale price for our common stock on The NASDAQ Capital Market was \$18.13 per share. The following table sets forth the range of high and low sales prices per share of our common stock as reported on The NASDAQ Capital Market for the period indicated.

Year Ended December 31, 2013	ŀ	ligh	1	Low
Third Quarter (from July 15, 2013 through September 30, 2013)	\$	17.24	\$	7.00
Fourth Quarter (from October 1, 2013 through October 23, 2013)	\$	20.90	\$	15.00

As of October 22, 2013, we had 14 stockholders of record, which excludes stockholders whose shares were held in nominee or street name by brokers. The actual number of common stockholders is greater than the number of record holders, and includes stockholders who are beneficial owners, but whose shares are held in street name by brokers and other nominees. This number of holders of record also does not include stockholders whose shares may be held in trust by other entities.

#### **DIVIDEND POLICY**

We have never declared or paid any cash dividends on our common 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. Any future determination related to our dividend policy will be made at the discretion of our Board of Directors and will depend upon, among other factors, our results of operations, financial condition, capital requirements, contractual restrictions, business prospects and other factors our Board of Directors may deem relevant.

#### CAPITALIZATION

The following table sets forth our cash, cash equivalents and marketable securities, and our capitalization as of June 30, 2013:

on an actual basis; and

on a pro forma basis to reflect the sale by us of 3,250,000 shares of our common stock in this offering at the public offering price of \$17.50 per share, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us.

You should read this table together with "Management's Discussion and Analysis of Financial Condition and Results of Operations" and our consolidated financial statements and the related notes appearing elsewhere in this prospectus.

	AC	AS OF JUNE 30, 2013 PRO ACTUAL FORMA (unaudited, in thousands, except share data)		
Cash, cash equivalents and marketable securities	\$	20,256 73,219		
Preferred stock; \$0.001 par value per share: 10,000,000 shares authorized, none issued and outstanding, actual; 10,000,000 shares authorized, none issued and outstanding, pro forma Stockholders' deficit: Common stock; \$0.001 par value per share: 100,000,000 shares authorized, 9,957,725 shares issued and outstanding, actual; 100,000,000 shares authorized, 13,207,725 shares issued and outstanding, pro forma Additional paid-in capital Accumulated other comprehensive income Accumulated deficit		10 154,469 9,520 (157,762)	13 207,428 9,520 (157,762)	
Total stockholders' equity		6,237	59,200	
Total capitalization	\$	6,237	59,200	

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The number of shares of common stock shown as issued and outstanding on a pro forma basis in the table is based on the number of shares of our common stock outstanding as of June 30, 2013, and excludes:

473,195 shares of common stock issuable upon the exercise of outstanding stock options as of June 30, 2013, at a weighted average exercise price of \$13.09 per share;

2,733,445 shares of common stock issuable upon the exercise of outstanding warrants as of June 30, 2013, at a weighted average exercise price of \$7.56 per share;

624,249 shares of common stock reserved for future issuance under the 2013 Plan as of June 30, 2013; and

300,000 shares of common stock reserved for future issuance under the ESPP as of June 30, 2013.

#### DILUTION

Investors purchasing shares of our common stock in this offering will experience immediate and substantial dilution in the pro forma net tangible book value of their shares of common stock. Dilution in pro forma net tangible book value represents the difference between the public offering price per share and the pro forma net tangible book value per share of our common stock immediately after the offering.

The historical net tangible book value of our common stock as of June 30, 2013 was \$6.2 million, or \$0.63 per share. Historical net tangible book value per share of our common stock represents our total tangible assets (total assets less intangible assets) less total liabilities divided by the number of shares of common stock outstanding as of that date.

After giving effect to (1) the issuance of 3,250,000 shares of common stock in this offering and (2) the receipt of the net proceeds from the sale of 3,250,000 shares of common stock in this offering at the public offering price of \$17.50 per share, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us, our net tangible book value as of June 30, 2013 would have been approximately \$59.2 million, or \$4.48 per share of common stock. This represents an immediate increase in net tangible book value of \$3.85 per share to existing stockholders and an immediate dilution of \$13.02 per share to new investors purchasing our common stock in this offering.

The following table illustrates this dilution on a per share basis to new investors:

Public offering price per share		\$ 17.50
Historical net tangible book value per share as of June 30, 2013	\$ 0.63	
Increase in net tangible book value per share attributable to new investors purchasing shares in this offering	3.85	
As adjusted net tangible book value per share after giving effect to this offering		4.48
Dilution per share to new investors participating in this offering		\$ 13.02
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If the underwriters exercise their option in full to purchase an additional 487,500 shares of common stock in this offering, the as adjusted net tangible book value per share after the offering would be \$4.91 per share, the increase in the net tangible book value per share to existing stockholders would be \$4.28 per share and the dilution to new investors purchasing our common stock in this offering would be \$12.59 per share.

The above discussion and table are based on 9,957,725 shares of common stock outstanding as of June 30, 2013, which does not include:

473,195 shares of common stock issuable upon the exercise of outstanding stock options as of June 30, 2013, at a weighted average exercise price of \$13.09 per share;

2,733,445 shares of common stock issuable upon the exercise of outstanding warrants as of June 30, 2013, at a weighted average exercise price of \$7.56 per share;

624,249 shares of common stock reserved for future issuance under the 2013 Plan as of June 30, 2013; and

300,000 shares of common stock reserved for future issuance under the ESPP as of June 30, 2013.

To the extent that outstanding exercisable options or warrants are exercised, you may experience further dilution. If all outstanding exercisable options and warrants with exercise prices below \$17.50 per share were exercised, our as adjusted net tangible book value as of June 30, 2013 (calculated on the basis of the

assumptions set forth above) would have been approximately \$93.0 million, or approximately \$5.51 per share, causing immediate dilution of \$11.99 per share to new investors purchasing shares in this offering.

In addition, we may choose to raise additional capital due to market conditions or strategic considerations, even if we believe we have sufficient funds for our current or future operating plans. To the extent that we raise additional capital by issuing equity securities or convertible debt, your ownership will be further diluted.

Share reserves for our share-based compensation plans will also be subject to automatic annual increase in accordance with the terms of the plans. To the extent that new options are issued under our share-based compensation plans or we issue additional shares of common stock under the ESPP in the future, there will be further dilution to investors participating in this offering.

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#### SELECTED CONSOLIDATED FINANCIAL DATA

The following selected consolidated financial data should be read together with our consolidated financial statements and accompanying notes and "Management's Discussion and Analysis of Financial Condition and Results of Operations" appearing elsewhere in this prospectus. The selected consolidated 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 the results that may be expected in the future and results of interim periods are not necessarily indicative of the results that may be expected in the future and results of interim periods are not necessarily indicative of the results that may be expected from our audited consolidated financial statements included elsewhere in this prospectus. The selected statement of operations data for the years ended December 31, 2011 and 2012 and the selected balance sheet data as of December 31, 2011 and 2012 are derived from our audited consolidated financial statements included elsewhere in this prospectus. The selected statement of operations data for the six months ended June 30, 2012 and 2013 and the selected balance sheet data as of June 30, 2013 are derived from our unaudited condensed consolidated financial statements included elsewhere in this prospectus. The unaudited condensed consolidated financial statements included consolidated financial statements included in this prospectus and include, in the opinion of management, all adjustments, consisting only of normal recurring adjustments, necessary for the fair presentation of the financial information in those statements.

Consolidated Statements of Operations and Comprehensive Loss	DECEMB 2011		in thousands, except		x 31, ENDED JUI 012 2012 (unaudit		JNE 30, 2013 ited)
Revenue							
Research collaborations and contract revenues	\$	811	\$		\$	\$	
License and up-front fees		2,333					
Total revenue		3,144					
Expenses							
Research and development, net		8,891		15,081		5,856	9,985
General and administrative		4,340		5,394		2,302	4,906
Total operating expenses		13,231		20,475		8,158	14,891
Loss from operations		(10,087)	(	20,475)		(8,158)	(14,891)
Other income, net		309		228		138	2,722
Loss before income taxes		(9,778)	(	20,247)	(	(8,020)	(12,169)
Income tax expense				39		13	60
Net loss and comprehensive loss for the period		(9,778)	(	20,286)		(8,033)	(12,229)
Basic and diluted net loss per share <sup>(1)</sup>	\$	(1.98)	\$	(3.00)	\$	(1.26) \$	(1.23)
Weighted average number of shares used in computing net loss per share, basic and							
diluted <sup>(1)</sup>	4,	944,184	6,7	62,985	6,35	58,266	9,957,725

(1)

See Note 16 to our audited consolidated financial statements and Note 11 to our unaudited condensed consolidated financial statements appearing elsewhere in this prospectus for an explanation of the method used to calculate the basic and diluted net loss per share and the weighted average number of shares used in computing the share and per share data.

### Explanation of Responses:

Consolidated Balance Sheet Data		DECEM 2011	BE	R 31, 2012		JNE 30, 2013 audited)	
			(in t	housands)	)		
Cash, cash equivalents and marketable securities	\$	28,445	\$	36,983	\$	20,256	
Working capital		26,711		33,989		5,644	
Total assets		31,082		39,801		23,040	
Accumulated deficit		(120,205)		(140,491)		(157,762)	
Total stockholders' equity		27,305		34,416		6,237	
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#### MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

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

We were incorporated under the laws of the State of Delaware on April 29, 2013. On May 8, 2013, our Board of Directors approved and we entered into an arrangement agreement with MethylGene Inc., a corporation incorporated under the Canada Business Corporations Act, or MethylGene Canada. Subject to the terms and conditions of the arrangement agreement, the shareholders of MethylGene Canada received one share of our common stock in exchange for every 50 common shares of MethylGene Canada, which had the effect of a 50-for-1 reverse split of the common shares pursuant to a court-approved plan of arrangement under Section 192 of the Canada Business Corporations Act. Such transaction is referred to herein as the Arrangement. In addition, all outstanding options and warrants to purchase common shares of MethylGene Canada became exercisable on a 50-for-1 basis for shares of our common stock, and a proportionate adjustment was made to the exercise price or conversion price, as applicable. Upon consummation of the Arrangement on June 28, 2013, MethylGene Canada became our wholly-owned subsidiary. As a result, the discussion contained in this Management's Discussion and Analysis of Financial Condition and Results of Operations, including the financial information and related disclosure, reflect the consolidated operations of MethylGene Canada.

We are a holding company whose only asset is stock of MethylGene Canada. To date we have conducted virtually all of our business operations though MethylGene Canada and its wholly-owned subsidiary, MethylGene US Inc.

Our historical functional currency was Canadian dollars as of December 31, 2012. Effective January 1, 2013, our functional currency is U.S. dollars. Our reporting currency is U.S. dollars and prior to January 1, 2013, for presentation purposes, assets and liabilities have been translated to U.S. dollars at exchange rates at the reporting date. Income and expenses have been translated to U.S. dollars at the average exchange rate for the period in which the transactions occurred. Equity transactions have been translated at the spot exchange rates on the date the transactions occurred. Exchange rate differences are recognized in a separate component of stockholders' equity titled accumulated other comprehensive income.

#### Overview

We are a clinical-stage biopharmaceutical company focused on developing a pipeline of targeted oncology products. We focus our development programs on drugs intended to treat specific subsets of cancer patients with unmet needs. Our pipeline consists of three product candidates: MGCD265, MGCD516 and mocetinostat. MGCD265 and MGCD516 are orally-bioavailable, multi-targeted kinase inhibitors with distinct target profiles that are in development to treat patients with NSCLC and other solid tumors. MGCD265 is in Phase 1/2 clinical development and MGCD516 is in advanced preclinical development, with Phase 1 clinical development anticipated to begin in the first half of 2014. Mocetinostat is an orally-bioavailable, spectrum-selective HDAC inhibitor for the first line treatment of patients with MDS. We are planning to initiate a Phase 3 clinical trial of mocetinostat in the second half of 2014.

In addition to our core programs, we also have collaborations with Otsuka Pharmaceutical Co. Ltd., or Otsuka, and EnVivo Pharmaceuticals, Inc., or EnVivo, for other non-oncology pipeline programs.

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We have not generated any revenue from product sales. To date, we have funded our operations primarily through the sale of our common stock and through up-front payments, research funding and milestone payments from our collaboration arrangements.

We have incurred losses in each year since our inception. Our net losses were \$12.2 million for the six months ended June 30, 2013, and \$20.3 million and \$9.8 million for the years ended 2012 and 2011, respectively. As of June 30, 2013, we had an accumulated deficit of \$157.8 million. Substantially all of our operating losses resulted from expenses incurred in connection with our product development programs, our research activities and general and administrative costs associated with our operations.

We expect to continue to incur significant expenses and increasing operating losses for at least the next several years. In the near term, we anticipate that our expenses will increase as we:

advance our two lead kinase programs, MGCD265 and MGCD516 in development for the treatment of solid tumors;

advance mocetinostat, our later stage drug candidate in development for the treatment of hematologic malignancies;

evaluate opportunities for the expansion of our oncology portfolio, including evaluating and possibly executing in-licensing and partnering transactions;

continue our translational science research efforts;

maintain, expand and protect our intellectual property portfolio; and

provide general and administrative support for our operations.

To fund future operations we will need to raise additional capital. The amount and timing of future funding requirements will depend on many factors, including the timing and results of our ongoing development efforts, the potential expansion of our current development programs, potential new development programs and related general and administrative support. We anticipate that we will seek to fund our operations through public or private equity or debt financings or other sources, such as potential collaboration agreements. We cannot assure you that anticipated additional financing will be available to us on favorable terms, or at all. Although we have previously been successful in obtaining financing through our equity securities offerings, there can be no assurance that we will be able to do so in the future.

On October 1, 2013, we announced that we expect to terminate approximately 27 employees, or approximately 75% of our total workforce, in connection with the closure of our Montreal, Quebec and Princeton, New Jersey offices. The offices are being closed due to the consolidation of our operations to our San Diego facility. We plan to partially offset this reduction in force by hiring additional personnel in the San Diego facility and by engaging third-party services providers to perform certain functions. We expect the terminations and office closures to be substantially completed by March 31, 2014. We estimate that we will incur pre-tax charges of approximately \$1.5 million relating to the office closures, consisting of approximately \$0.9 million in one-time cash severance payments and related benefits, approximately \$0.2 million in office closing costs, and approximately \$0.4 million in asset impairment charges. We expect to recognize substantially all of the pre-tax charges by the first quarter of 2014. Approximately \$1.1 million of these charges are expected to result in future cash expenditures. The numbers set forth above are preliminary and may change as a result of a number of factors, including the timing of the terminations and office closures.

#### **Financial Operations Overview**

#### Revenue

To date, we have not generated any revenue from product sales and do not expect to do so for a number of years. Revenue to date have been generated substantially from our research collaborations and license agreements. Since our inception through June 30, 2013, we have generated \$92.5 million in revenue under our various collaboration arrangements. We do not anticipate significant revenue from our existing collaboration arrangements in the foreseeable future. We may never generate revenue from MGCD265,

MGCD516 or mocetinostat, as we may never succeed in obtaining regulatory approval or commercializing any of these product candidates.

#### **Research and Development Expenses**

Research and development expenses consist primarily of salaries, benefits, stock-based compensation and related personnel costs for our employees, fees paid to external service providers such as CROs and contract manufacturing organizations related to clinical trials, contractual obligations for clinical development, clinical sites, manufacturing and scale-up, formulation of clinical drug supplies, and costs for facilities and amortization of equipment. We expense research and development expenses as incurred. We account for nonrefundable advance payments for goods and services that will be used in future research and development activities as expense when the services have been performed or when the goods have been received. Since our inception, we have spent a total of \$221.8 million in research and development programs we are unable to estimate with any certainty the costs we will incur in the continued development of MGCD265, MGCD516 and mocetinostat. We expect that our research and development expenses may increase if we are successful in advancing MGCD265, MGCD516, mocetinostat or any of our preclinical programs into advanced stages of clinical development. The process of conducting clinical trials necessary to obtain regulatory approval and manufacturing scale-up to support expanded development and potential future commercialization is costly and time consuming. Any failure by us or delay in completing clinical trials, manufacturing scale up or in obtaining regulatory approvals could lead to increased research and development expense and, in turn, have a material adverse effect on our results of operations.

Our recent historical research and development efforts have been focused on MGCD265 for oncology and MGCD290 for antifungal indications. In future periods, we intend to focus our research and development efforts on our oncology programs, including our two lead kinase programs, MGCD265 and MGCD516, and our HDAC inhibitor, mocetinostat. The following table summarizes our research and development expenses, in thousands:

	YEARS ENDED DECEMBER 31, 2012 2011					SIX MONTH ENDED JUNE 30, 2013 201			
Third-party clinical development costs:	4	2012	4	2011		2013		2012	
MGCD265	\$	6.377	\$	3,342	\$	3.917	\$	2,476	
MGCD290	-	3,054	Ŧ	1,402	Ŧ	998	Ŧ	1,174	
mocetinostat		52		113		615		32	
MGCD516						457			
Total third-party clinical development costs		9,483		4,857		5,987		3,682	
Internal clinical development costs		3,609		2,196		2,820		2,193	
Total clinical development		13,092		7,053		8,807		5,875	
Non-clinical research and development		3,668		2,732		1,660		1,374	
Research and development, gross		16,760		9,785		10,467		7,249	
Less: Investment tax credits		(1,675)		(894)		(482)		(1,393)	
Research and development, net	\$	15,081	\$	8,891	\$	9,985	\$	5,856	
•									

We reported data from a recently completed Phase 2 trial for MGCD290 in vulvovaginal candidiasis in March 2013. In this study, MGCD290 failed to improve the rate of therapeutic cure. While we continue to analyze the data and results of that trial, at the present time we do not expect to prioritize development of MGCD290 internally and we will explore whether there may be external interest in the program. Our

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development program for MGCD265 is in Phase 1/2 clinical development, MGCD516 is in advanced preclinical development and we are currently in Phase 2 clinical development of mocetinostat. Given this early stage of clinical development and the significant risks and uncertainties inherent in the clinical development, manufacturing and regulatory approval process, we are unable to estimate with any certainty the time or cost to complete the development of MGCD265, MGCD516 and mocetinostat. Clinical development timelines, the probability of success and development costs can differ materially from expectations and results from our clinical trials may not be favorable. If we are successful in progressing MGCD265, MGCD516 into later stage development, we will require additional capital; however, while we are currently focused on advancing MGCD265, MGCD516 and mocetinostat, the amount and timing of our future research and development expenses will depend on the preclinical and clinical success of both our current development activities and potential development of new product candidates, as well as ongoing assessments of the commercial potential of such activities. We expect our research and development expenses to increase in the future as we advance our product candidates in clinical development.

#### General and Administrative Expenses

General and administrative expenses consist primarily of salaries and related benefits, including stock-based compensation related to our executive, finance, business development and support functions. Other general and administrative expenses include rent and utilities, travel expenses and professional fees for auditing, tax and legal services. We expect that general and administrative expenses may increase in the future as we expand our operating activities. In addition, general and administrative costs in 2013 are expected to reflect increased costs associated with our listing as a publicly traded company in the United States and our associated transition to becoming a Delaware corporation. We anticipate incurring one-time costs associated with the listing of our shares of common stock on The NASDAQ Capital Market and the transition to becoming a Delaware corporation of approximately \$1.5 million to \$2.0 million in 2013, consisting primarily of legal and accounting fees, of which \$1.4 million was incurred in the six months ended June 30, 2013. Incremental recurring external costs associated with becoming a publicly traded company in the United States are estimated to be approximately \$0.5 million per year, consisting primarily of increased legal, accounting and insurance costs.

#### Other Income, Net

Other income consists primarily of interest income, foreign exchange gains and losses and fair value gains and losses on our warrant liability.

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

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

As of June 30, 2013, there were no significant changes in critical accounting policies from those at December 31, 2012. The financial information as of June 30, 2013 should be read in conjunction with the financial statements for the year ended December 31, 2012, and the related notes thereto, contained elsewhere in this prospectus.

#### **Revenue Recognition**

We recognize revenue from various research, collaboration and license agreements which may include multiple elements, such as when the contracted services are performed or when milestones are achieved, in accordance with the terms of the specific agreements. Up-front payments for the use of technology where further services are to be provided or fees received upon the signing of research agreements are recognized over the period of performance of the related activities, and as such, require estimates. Up-front licensing revenue is deferred and recognized over the term during which we maintain substantive contractual obligations, which may also involve estimates from management. In the event the substantive obligation changes, an appropriate adjustment will be made to the amortization of deferred revenue. Amounts received

in advance of revenue recognition are included in deferred revenue. Milestone payments are recognized as they are earned. Revenue that is recognized but has not been invoiced to partners is recorded as unbilled revenue.

#### Accrued Research and Development Expenses

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 expense accruals for clinical trials are based on estimates of the fees associated with services provided by clinical trial investigational sites and CROs. Payments under some of the contracts we have with such parties depend on factors such as successful enrollment of patients, site initiation and the completion of clinical trial milestones. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If possible, we obtain information regarding unbilled services directly from these service providers. However, we may be required to estimate these services based on other information available to us. If we underestimate or overestimate the activity or fees associated with a study or service at a given point in time, adjustments to research and development expenses may be necessary in future periods. Historically, our estimated accrued expenses have approximated actual expense incurred. Subsequent changes in estimates may result in a material change in our accruals.

#### **Government** Assistance

We incur research and development expenses, which are eligible for refundable investment tax credits, or ITCs. The ITCs recorded are based on our estimates of amounts expected to be recovered and are subject to an audit by the taxation authorities, which may result in material differences. We claimed refundable ITCs from the provincial tax authority in 2012 and 2011. As we are a public company, federal ITCs are not refundable.

#### Stock-Based Compensation

We account for stock-based compensation expense related to stock options granted to employees and members of our Board of Directors by estimating the fair value of each stock option at the date of the grant using the Black-Scholes option-pricing model. For awards subject to time-based vesting conditions, we recognize stock-based compensation expense ratably over the vesting period of the options. Awards with graded vesting are considered multiple awards for fair value measurement and stock-based compensation calculation. In determining the expense, we deduct the number of options that are expected to be forfeited at the time of a grant and revise this estimate, if necessary, in subsequent years if actual forfeitures differ from those estimated.

We recognized stock-based compensation expense as follows (in thousands):

	~	X MO ENI JUN	DEL		EAR E ECEMI			
	20	)13	20	012	2	012	20	011
Research and development	\$	67	\$	341	\$	817	\$	268
General and administrative		178		311		1,192		672
	\$	245	\$	652	\$	2,009	\$	940

#### Key assumptions

We utilize the Black-Scholes option-pricing model, which requires the input of highly subjective assumptions, including the risk-free interest rate, the expected dividend yield of our common stock, the expected volatility of the price of our common stock and the expected life of the option. These estimates involve inherent risk and uncertainties and the application of management's judgment. If factors change and different assumptions are used, our stock-based compensation expense could be materially different in the future.

The fair value of options granted is estimated at the date of grant using the Black-Scholes option-pricing model and the following assumptions:

	SIX MON ENDI JUNE	ED	YEAR EN DECEMB	
Weighted Average	2013	2012	2012	2011
Risk-free interest rate	1.52%	1.20%	1.18%	2.04%
Dividend yield	0%	0%	0%	0%
Volatility factor	104.24%	117.14%	116.43%	116.79%
Expected life (years)	7	4.36	4.42	4.27

These assumptions are estimated as follows:

*Risk-free interest rate:* We utilize the risk-free interest rate for periods equal to the expected life of share options based on the Canadian Treasury Yield in effect at the time of the grant.

*Expected dividend yield:* We base the expected dividend yield assumption on the fact that we have never paid cash dividends and have no present intention to pay cash dividends. Consequently, we used an expected dividend of zero.

*Expected volatility:* The expected stock price volatility is estimated by taking the average historic price volatility of our shares of common stock based on the grant date and on daily pricing observations over a period equivalent to the expected term of the stock option grants.

*Expected life:* The expected life represents the period of time that the options are expected to be outstanding based on management's best estimates on its current programs' success and milestones to be achieved within the term of the options granted, as well as consideration of historical data.

*Pre-vesting forfeitures:* Estimates of pre-vesting forfeitures are based on historical experience. The difference between actual forfeitures and estimated forfeitures is recognized through a cumulative catch-up adjustment in the period of change and will also impact the amount of compensation expense to be recognized in future periods.

#### **Functional Currency**

Historically, our functional currency has been the Canadian dollar and the functional currency of our subsidiaries, MethylGene Canada and MethylGene US Inc., has also been the Canadian dollar.

Management undertook a detailed review of the appropriateness of the status of our functional currency by operating department for 2013. As we do not have revenue, the primary factor in determining functional currency relates to the currency in which we incur most of our expenditures. Based on the projected level of spending on clinical trials, which are predominantly denominated in U.S. dollars, coupled with the increase in U.S.-based employees, we concluded that spending in U.S. dollars will exceed that in Canadian dollars for 2013 and onwards. As we do not foresee a reversal of this trend, management has transitioned the functional currency to the U.S. dollar effective January 1, 2013. Management undertakes a detailed review of the appropriateness of the status of our functional currency on a quarterly basis. Our reporting currency is U.S. dollars.

In 2011 and 2012, we issued common stock warrants in connection with the issuance of common stock through private placements with exercise prices denominated in Canadian dollars. Upon the issuance of these common stock warrants, we allocated the net proceeds to common stock and warrants based on their relative fair values, and calculated the fair value of the issued common stock warrants utilizing the Black-Scholes option-pricing model. The allocated fair value was then recorded as warrants within stockholders' equity on the consolidated balance sheet. The fair value was not remeasured in periods subsequent to the date of issuance.

### Explanation of Responses:

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The change in our functional currency to the U.S. dollar effective January 1, 2013 changed how we account for our warrants which have exercise prices denominated in Canadian dollars. Upon the change in functional currency, we classified these warrants as a current liability and recorded a warrant liability of \$16.2 million which represented the fair market value of the warrants at that date in accordance with Accounting Standards Codification, or ASC, 815, "*Derivatives and Hedging*." The initial fair value recorded as warrants within stockholders' equity of \$11.2 million was reversed. The change in fair value related to periods prior to January 1, 2013 of \$5.0 million was recorded as an adjustment to accumulated deficit. At each reporting period subsequent to January 1, 2013, we will adjust the fair value of the warrant liability and any corresponding increase or decrease to the warrant liability will be recorded as a component of other income (expense) on the consolidated statement of operations and comprehensive loss. The estimated fair value is determined using the Black-Scholes option-pricing model based on the estimated value of the underlying common stock at the valuation measurement date, the remaining contractual term of the warrant, risk-free interest rates, expected dividends and expected volatility of the price of the underlying common stock. The fair value of the warrant liability was \$12.2 million at June 30, 2013 and we recorded a gain of \$4.0 million for the six months ended June 30, 2013 which is included in other income in the consolidated statement of operations and comprehensive loss.

#### **Transactions and Balances**

Foreign currency transactions are translated into the functional currency using the exchange rates prevailing at the dates of the transactions. Foreign exchange gains and losses resulting from the settlement of foreign currency transactions and from the remeasurement of monetary assets and liabilities denominated in currencies other than our functional currency are recognized in other income (expense).

#### Net Operating Loss Carryforwards and Investment Tax Credits

As of December 31, 2012, we had Canadian net operating loss carryforwards of \$25.5 million for Canadian federal income tax purposes and \$25.7 million for provincial income tax purposes, both of which begin to expire in 2030.

As of December 31, 2012, we recorded Canadian provincial refundable ITCs of \$1.7 million as a reduction of research and development expenditures. In addition, we had Canadian federal non-refundable ITCs of \$3.0 million as at December 31, 2012, which may be utilized to reduce future federal income taxes payable. The non-refundable Canadian federal ITCs begin to expire in 2030.

#### **Recent Accounting Pronouncements**

See "Notes to Consolidated Financial Statements Note 3 Recent Accounting Pronouncements" contained elsewhere in this prospectus.

#### Jobs Act

In April 2012, the JOBS Act was signed into law. The JOBS Act contains provisions that, among other things, reduce certain reporting requirements for an "emerging growth company." As an "emerging growth company," we are electing not to take advantage of the extended transition period afforded by the JOBS Act for the implementation of new or revised accounting standards, and as a result, we will comply with new or revised accounting standards on the relevant dates on which adoption of such standards is required for non-emerging growth companies. Section 107 of the JOBS Act provides that our decision not to take advantage of the extended transition period is irrevocable. In addition, we are in the process of evaluating the benefits of relying on the other exemptions and reduced reporting requirements provided by the JOBS Act. Subject to certain conditions set forth in the JOBS Act, if as an "emerging growth company" we choose to rely on such exemptions, we may not be required to, among other things, (i) provide an auditor's attestation report on our system of internal controls over financial reporting pursuant to Section 404, (ii) provide all of the compensation disclosure that may be required of non-emerging growth public companies under the Dodd-Frank Wall Street Reform and Consumer Protection Act, (iii) comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory

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audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements (auditor discussion and analysis), and (iv) disclose certain executive compensation-related items such as the correlation between executive compensation and performance and comparisons of the Chief Executive Officer's compensation to median employee compensation. These exemptions will apply for a period of five years following the first sale of our common stock pursuant to an effective registration statement or until we no longer meet the requirements of being an "emerging growth company," whichever is earlier.

#### **Results of Operations**

#### Comparison of the Three Months Ended June 30, 2013 and 2012

The following table summarizes our results of operations for the three months ended June 30, 2013 and 2012 (in thousands):

		IREE M NDED J	 	In	crease
	2	2013	2012	(De	crease)
Research and development, net	\$	4,510	\$ 3,652	\$	858
General and administrative		2,382	1,082		1,300
Other (loss)/income, net		(1,079)	70		(1,149)

Research and Development Expenses

Our research and development efforts have been focused on MGCD265 for oncology and MGCD290 for antifungal indications. In future periods, we intend to focus our research and development efforts on oncology, including MGCD265, MGCD516 and mocetinostat. The following table summarizes our research and development expenses (in thousands):

Third-party clinical development costs:MGCD265\$ 2,046\$ 1,308MGCD290166639mocetinostat53218MGCD5163727Total third-party clinical development costs3,1161,965Internal clinical development costs1,1321,107Total clinical development4,2483,072Non-clinical research and development515671Research and development, gross4,7633,743Less: Investment tax credits(253)(91)		Eľ	IREE N NDED J 2013	JUN	
MGCD290166639mocetinostat53218MGCD516372372Total third-party clinical development costs3,1161,965Internal clinical development costs1,1321,107Total clinical development4,2483,072Non-clinical research and development515671Research and development, gross4,7633,743	Third-party clinical development costs:				
mocetinostat53218MGCD516372372Total third-party clinical development costs3,1161,965Internal clinical development costs1,1321,107Total clinical development4,2483,072Non-clinical research and development515671Research and development, gross4,7633,743	MGCD265	\$	2,046	\$	1,308
MGCD516372Total third-party clinical development costs3,1161,965Internal clinical development costs1,1321,107Total clinical development4,2483,072Non-clinical research and development515671Research and development, gross4,7633,743	MGCD290		166		639
Total third-party clinical development costs3,1161,965Internal clinical development costs1,1321,107Total clinical development4,2483,072Non-clinical research and development515671Research and development, gross4,7633,743	mocetinostat		532		18
Internal clinical development costs1,1321,107Total clinical development4,2483,072Non-clinical research and development515671Research and development, gross4,7633,743	MGCD516		372		
Internal clinical development costs1,1321,107Total clinical development4,2483,072Non-clinical research and development515671Research and development, gross4,7633,743					
Total clinical development4,2483,072Non-clinical research and development515671Research and development, gross4,7633,743	Total third-party clinical development costs		3,116		1,965
Non-clinical research and development515671Research and development, gross4,7633,743	Internal clinical development costs		1,132		1,107
Non-clinical research and development515671Research and development, gross4,7633,743					
Research and development, gross 4,763 3,743	Total clinical development		4,248		3,072
	Non-clinical research and development		515		671
	Research and development, gross		4,763		3,743
			(253)		(91)
Research and development, net\$ 4,510\$ 3,652	Research and development, net	\$	4,510	\$	3,652

Net research and development expenses were \$4.5 million for the three months ended June 30, 2013 compared to \$3.7 million for the same period in 2012. The increase primarily reflects increased third-party clinical development costs due to ongoing formulation development for MGCD265, costs associated with the preparation for initiating clinical development for mocetinostat in the fourth quarter of 2013, and costs

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associated with an IND for MGCD516, planned for the first half of 2014. Partially offsetting these increases were reduced costs for MGCD290, which we are no longer actively pursuing internally, and an increase in ITCs due to our higher level of investment in research and development activities.

#### General and Administrative Expenses

General and administrative expenses were \$2.4 million for the three months ended June 30, 2013 compared to \$1.1 million for the same period in 2012. The increase primarily reflects costs associated with the previously described Arrangement agreement and subsequent listing on The NASDAQ Capital Market, which was effective on July 15, 2013.

#### Other (Loss)/Income, Net

Other (loss)/income, net was a loss of \$1.1 million for the three months ended June 30, 2013 compared to income of \$70,000 for the same period in 2012. This increase primarily reflects a foreign exchange loss of \$754,000 as a result of transitioning to the U.S. dollar as the functional currency and a loss of \$385,000 due to the change in fair value of our warrant liability.

#### Comparison of the Six Months Ended June 30, 2013 and 2012

The following table summarizes the results of our operations for the six months ended June 30, 2013 and 2012 (in thousands):

	SIX MONTHS ENDED JUNE 30,						
	2	013		2012	In	crease	
Research and development, net	\$	9,985	\$	5,856	\$	4,129	
General and administrative		4,906		2,302		2,604	
Other income, net		2,722		138		2,584	

Research and Development Expenses

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

	E	SIX MO NDED J 2013	IUN	
Third-party clinical development costs:				
MGCD265	\$	3,917	\$	2,476
MGCD290		998		1,174
mocetinostat		615		32
MGCD516		457		
Total third-party clinical development costs:		5,987		3,682
Internal clinical development costs		2,820		2,193
Total clinical development		8,807		5,875
Non-clinical research and development		1,660		1,374
•				
Research and development, gross		10,467		7,249
Less: Investment tax credits		(482)		(1,393)
Research and development, net	\$	9,985	\$	5,856

Net research and development expenses were \$10.0 million for the six months ended June 30, 2013 compared to \$5.9 million for the same period in 2012. The increase primarily reflects the same factors that influenced similar increases in the three months ended June 30, 2013 discussed above as well as costs

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associated with management changes implemented in the first quarter of 2013 and a reduction in ITCs due to the fact that the prior year included a favorable adjustment of prior year calculations subsequent to the completion of an audit by the provincial tax authority.

#### General and Administrative Expenses

General and administrative expenses were \$4.9 million for the six months ended June 30, 2013 compared to \$2.3 million for the same period in 2012. The increase primarily reflects the same factors that influenced similar fluctuations in the three months ended June 30, 2013 as well as costs associated with management changes implemented in the first quarter of 2013.

#### Other Income, Net

Other income, net was \$2.7 million for the six months ended June 30, 2013 compared to \$138,000 for the same period in 2012. The increase primarily reflects a gain of \$4.0 million from the change in fair value of our warrant liability, partially offset by a foreign exchange loss of \$1.4 million primarily due to the transition to the U.S. dollar as the functional currency.

#### Comparison of the Years Ended December 31, 2012 and 2011

The following table summarizes the results of our operations for the years ended December 31, 2012 and 2011 (in thousands):

	YEAR I DECEM		Increase
	2012	2011	(Decrease)
Research collaborations and contract revenues	\$	\$ 811	\$ (811)
License and up-front fees		2,333	(2,333)
Research and development, net	15,081	8,891	6,190
General and administrative	5,394	4,340	1,054
Other income, net	228	309	(81)

#### Revenue

#### Research Collaborations and Contract Revenues

Research collaborations and contract revenues were \$0 in 2012, compared to \$811,000 in 2011. Research collaboration and contract revenues in 2011 reflect reimbursed development expenses from Otsuka of \$809,000. There were no revenues in 2012 as the research component of our collaboration agreement ended on June 30, 2011.

#### License and Up-front Fees

There were no license and up-front fees in 2012 compared to \$2.3 million in 2011. We had recorded license and up-front revenue in 2011 in connection with both the Otsuka and Taiho agreements (\$1.7 million and \$660,000, respectively). When our substantial obligations ended under both agreements, we amortized the remaining deferred revenue under the Otsuka and Taiho agreements in the second and fourth quarters of 2011, respectively.

#### Research and Development Expenses

Net research and development expenses were \$15.1 million in 2012 compared to \$8.9 million in 2011. The increase of \$6.2 million primarily reflects \$5.0 million of increased costs associated with the two ongoing Phase 1 clinical trials of MGCD265 and the recently completed Phase 2 clinical trial of MGCD290. The increase also reflects, to a lesser extent, \$1.8 million of increased employee expenses associated with the hiring of a Chief Medical Officer and several additional senior management staff during 2012. Partially offsetting these increased expenses was an increase in ITCs of \$0.8 million due primarily to a favorable adjustment of prior year calculations subsequent to the completion of an audit by the provincial tax authority.

#### General and Administrative Expenses

General and administrative expenses were \$5.4 million in 2012 compared to \$4.3 million in 2011. The increase of \$1.1 million primarily reflects an increase in employee expenses of \$1.0 million for costs associated with the resignation of our former Chief Executive Officer and the appointment of our current Chief Executive Officer.

#### Other Income, Net

Other income, net was \$0.2 million in 2012 compared to \$0.3 million in 2011. The decrease of \$0.1 million primarily reflects lower interest income of \$24,000 due to lower average cash balances in 2012 and 2011 and the unfavorable impact of foreign exchange rates between the U.S. dollar and Canadian dollar of \$56,000.

#### Liquidity and Capital Resources

To date, we have funded our operations primarily through the sale of our common stock and through up-front payments, research funding and milestone payments from our collaboration arrangements. Since inception, we have primarily devoted our resources to funding research and development programs, including discovery research, preclinical and clinical development activities.

#### **Going Concern**

As of June 30, 2013, substantial doubt exists over our ability to continue as a going concern. We believe that our cash, cash equivalents and marketable securities are sufficient to carry out our currently planned clinical development and operating plans into the second quarter of 2014, without considering the expansion of our development activities to include the development of our preclinical drug candidate MGCD516 and mocetinostat, our later-stage product candidate, or potential future financing. Our cash, cash equivalents, marketable securities and term deposits decreased by \$16.7 million in the six months ended June 30, 2013, reflecting a rate of negative cash flow per month of approximately \$2.8 million. Excluding non-recurring costs associated with recent management changes and costs associated with the previously described Arrangement agreement and listing on The NASDAQ Capital Market, our cash, cash equivalents, marketable securities and term deposits decreased by \$14.1 million in the six months ended June 30, 2013 reflecting a rate of negative cash flow per month of approximately \$2.4 million. While our rate of future negative cash flow per month will vary due to the timing of expenses incurred, at the current rate of negative cash flow per month we believe that our current cash, cash equivalents and marketable securities will enable us to complete Phase 1 development of MGCD265, which if successful would enable us to enter Phase 2 development. Our future cash requirements could increase if we decide to expand our research and development efforts beyond the currently planned development.

We have incurred operating losses in each year since our inception and we expect to continue to incur operating losses into the foreseeable future as we advance our two lead kinase inhibitors, MGCD265 and MGCD516, advance mocetinostat, our later-stage product candidate, evaluate opportunities for the potential expansion of our oncology pipeline and continue our research efforts. To fund future operations we will need to raise additional capital. The amount and timing of future funding requirements will depend on many factors including the timing and results of our ongoing development efforts, the potential expansion of our current development programs, potential new development programs and related general and administrative support. We anticipate that we will seek to fund our operations through public or private equity or debt financings or other sources, such as potential collaboration agreements. Additional financing may not be available to us on favorable terms, or at all. Although we have previously been successful in obtaining financing through our equity securities offerings, we may not be able to do so in the future. If we are not able to secure adequate additional financings we may be forced to make reductions in spending and/or liquidate assets where possible. Any of these actions could harm our business and our results of operations.

At June 30, 2013, we had \$20.6 million of cash, cash equivalents and marketable securities, and restricted cash and marketable securities compared to \$37.4 million at December 31, 2012. At



September 30, 2013, we estimate that we had approximately \$15.0 million in cash, cash equivalents and marketable securities. This amount is preliminary and subject to change.

We believe that our current cash and cash equivalents and marketable securities will sustain our currently planned operations into the second quarter of 2014, and that based on our plans as described in this prospectus, our current cash, cash equivalents and marketable securities, together with the net proceeds from this offering, will sustain our operations through the end of 2015. Pursuant to our current plans, we do not anticipate initiating a Phase 3 clinical trial with mocetinostat absent additional financing beyond this offering or the establishment of a collaboration for late-stage development.

#### Cash Flows for the Six Months Ended June 30, 2013 and 2012

#### **Operating Activities**

Cash used for operating activities for the six months ended June 30, 2013 was \$16.5 million compared to \$7.3 million for the same period in 2012. The increase relates primarily to the increased operating costs in the first six months of 2013 compared to the same period in 2012, as discussed above.

#### Investing Activities

Investing activities consist primarily of purchases, sales and maturities of marketable securities and term deposits and purchases of property and equipment. Investing activities provided cash of \$7.9 million and \$5.4 million for the six months ended June 30, 2013 and 2012, respectively. We acquired \$156,000 of property and equipment in the six months ended June 30, 2013 compared to \$51,000 in the six months ended June 30, 2012. This increase reflects higher capital expenditures for information technology.

#### Financing Activities

Financing activities consist primarily of net proceeds from the sale of common stock and warrants and proceeds from the exercise of stock options and warrants. There were no financing activities for the six months ended June 30, 2013. We used \$16,000 of cash for reorganization costs for the six months ended June 30, 2012.

As of June 30, 2013 we had restricted cash equivalents and marketable securities of \$363,000, compared to \$374,000 at December 31, 2012. We expect the restricted cash equivalents and marketable securities to reduce to \$78,000 by the end of November 2013 related to letters of credit underlying corporate credit cards.

#### Cash Flows for the Years Ended December 31, 2012 and 2011

#### **Operating Activities**

Cash used for operating activities for 2012 was \$16.9 million, compared to \$11.6 million in 2011, an increase of \$5.3 million. This increase relates primarily to lower revenue from collaborative arrangements and higher clinical development costs in 2012 compared to 2011.

#### **Investing Activities**

Investing activities consist primarily of purchases, sales and maturities of marketable securities and purchases of property and equipment. Investing activities provided cash of \$326,000 for 2012 and used cash of \$19.2 million for 2011. We acquired \$230,000 of property and equipment during 2012 compared to \$110,000 in 2011, an increase of \$120,000. The increase relates primarily to higher spending on information technology equipment along with some office equipment for our U.S. subsidiary located in Princeton, New Jersey.

#### Financing Activities

Financing activities consist primarily of net proceeds from the sale of common stock and warrants and proceeds from the exercise of stock options and warrants. Financing activities generated cash flows of \$24.8 million in 2012 compared to \$33.6 million in 2011, a decrease of \$8.8 million. Cash flows from financing activities included net proceeds from private placements of our common stock of \$24.8 million and \$33.6 million in 2012 and 2011, respectively.

#### **Off-Balance Sheet Arrangements**

During 2011 and 2012 and the six months ended June 30, 2013, we did not have any off-balance sheet arrangements (as defined by applicable SEC regulations) that are reasonably likely to have a current or future material effect on our financial condition, results of operations, liquidity, capital expenditures or capital resources.

#### **Internal Control Over Financial Reporting**

Pursuant to Section 404(a) of the Sarbanes-Oxley Act, commencing the year following our first annual report required to be filed with the SEC, our management will be required to report on the effectiveness of our internal control over financial reporting. While we have been subject to similar requirements pursuant to applicable Canadian requirements for companies listed on the TSX, the rules governing the standards that must be met for management to assess our internal control over financial reporting are complex and require significant documentation, testing and possible remediation. To comply with the requirements of being a reporting company under the Exchange Act, we may need to upgrade our systems, including information technology, implement additional financial and management controls, reporting systems and procedures and hire additional accounting and finance staff.

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#### BUSINESS

#### Overview

We are a clinical-stage biopharmaceutical company focused on developing a pipeline of targeted oncology products. We focus our development programs on drugs intended to treat specific subsets of cancer patients with unmet needs. Our pipeline consists of three product candidates: MGCD265, MGCD516 and mocetinostat. MGCD265 and MGCD516 are orally-bioavailable, multi-targeted kinase inhibitors with distinct target profiles that are in development to treat patients with non-small cell lung cancer, or NSCLC, and other solid tumors. MGCD265 is in Phase 1/2 clinical development and MGCD516 is in advanced preclinical development, with Phase 1 clinical development anticipated to begin in the first half of 2014. Mocetinostat is an orally-bioavailable, spectrum-selective histone deacetylase, or HDAC, inhibitor for the first line treatment of patients with myelodysplastic syndromes, or MDS. We are planning to initiate a Phase 3 clinical trial of mocetinostat in the second half of 2014.

We believe that an increased understanding of the genomic factors that drive tumor cell growth can lead to the development of cancer drugs with increased efficacy while reducing side effects. We are leveraging this knowledge to develop targeted cancer therapies to address unmet needs in selected cancer patient populations. Our novel kinase inhibitors target specific mutations present only in cancer cells, and mocetinostat acts through epigenetic mechanisms important in treating certain cancers. We plan to identify additional opportunities by leveraging our deep scientific understanding of molecular drug targets and mechanisms of resistance and potentially in-licensing promising, early-stage novel drug candidates.

Our three product candidates are as follows:

**MGCD265** is an orally-bioavailable, potent, small molecule multi-targeted kinase inhibitor of Met, Axl and VEGFRs. MGCD265 is in development for the treatment of solid tumors, with an initial focus on NSCLC and squamous cell carcinoma of the head and neck, or HNSCC. We have conducted single agent and combination dose escalation trials in 252 patients, with acceptable tolerability and promising early signs of clinical efficacy in patients with advanced solid tumors who have failed standard therapies. Our preclinical studies, in a variety of in vivo tumor models, have suggested that MGCD265 has relatively low toxicity and appears more potent than some of the leading approved kinase inhibitors, including Nexavar, Sutent and Xalkori. We have developed new formulations of MGCD265 designed to increase plasma exposure, improve the degree of target inhibition and increase the likelihood of seeing single agent clinical activity. Assuming one or more of the new formulations achieve sufficient patient exposure in ongoing studies, we intend to select one of the new formulations for introduction into ongoing dose escalation trials with the goal of identifying the maximum tolerated dose, or MTD, by early 2014. Following identification of the MTD, we plan to initiate dose expansion cohorts in patients selected for certain biomarkers.

**MGCD516** is an orally-bioavailable, potent, small molecule multi-targeted kinase inhibitor of RET, TRK, DDR and EphRs, as well as Met, Axl and VEGFRs, in development for the treatment of solid tumors. We plan to focus on solid tumors expressing RET, TRK and DDR, initially in NSCLC, and we plan to evaluate other tumor types where the profile of MGCD516 would suggest activity. MGCD516 is in advanced preclinical development. We plan to file an investigational new drug application, or IND, with the U.S. Food and Drug Administration, or FDA, and initiate a Phase 1 clinical trial of this product candidate in the first half of 2014, and identify the MTD and initiate expansion cohorts in patients selected for certain biomarkers by the end of 2014.

**Mocetinostat** is an orally-bioavailable, spectrum-selective HDAC inhibitor for which we plan to conduct a dose confirmation trial starting in the fourth quarter of 2013, with the goal of initiating a Phase 3 clinical trial in the second half of 2014. We have completed 13 clinical trials which enrolled 437 patients with a variety of hematologic malignancies and solid tumors. We intend to seek a Special Protocol Assessment, or SPA, from the FDA prior to the initiation of our planned Phase 3 trial. This trial will evaluate mocetinostat for the first line treatment of patients with MDS in

combination with Vidaza, a hypomethylating agent, or HMA. We believe that mocetinostat has the potential to be the first HDAC inhibitor to market for this indication.

Our management team has extensive experience in leading the discovery and development of targeted oncology therapies. Our President and Chief Executive Officer, Charles M. Baum, M.D., Ph.D., was Senior Vice President for Biotherapeutic Clinical Research within Pfizer Inc.'s Worldwide Research and Development division and previously Head of Oncology Development for Pfizer. Prior to Pfizer, he was also responsible for the development of several oncology compounds at Schering-Plough Corporation (acquired by Merck & Co., Inc., or Merck). Our Chief Medical and Development Officer, Isan Chen, M.D., was Chief Medical Officer of Aragon Pharmaceuticals, Inc., which was acquired by Johnson & Johnson in 2013. At Aragon Pharmaceuticals, Dr. Chen was responsible for the clinical development strategy of all of the company's programs, including prostate and breast cancer. Our Vice President of Research, James Christensen, Ph.D., was previously the Senior Director of Oncology Precision Medicine at Pfizer, where he was responsible for strategy and translational research for the entire Pfizer oncology drugs including Inlyta, Sutent, Temodar and Xalkori. In addition, our Executive Vice President and Chief Operations Officer, Mark J. Gergen, has experience in operations, finance, strategy and corporate development and was previously Senior Vice President of Corporate Development at Amylin Pharmaceuticals, Inc. until its acquisition by Bristol-Myers Squibb Inc. in 2012.

We have a collaboration agreement with Taiho Pharmaceutical Co. Ltd., or Taiho, covering mocetinostat in certain Asian territories and we own all rights to mocetinostat outside of those territories.

#### **Our Strategy**

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

**Develop a pipeline of targeted cancer therapies.** We believe that an increased understanding of the genomic factors that drive tumor cell growth can lead to the development of cancer drugs with increased efficacy while reducing side effects. We are leveraging this knowledge to develop targeted cancer therapies to address unmet needs in specific cancer populations. Our current pipeline is comprised of novel kinase inhibitors that target specific mutations present only in cancer cells and one of the most advanced epigenetic therapies in development. We plan to identify additional targets by leveraging our deep scientific understanding of molecular drug targets and mechanisms of resistance and potentially in-licensing promising, early-stage novel drug candidates.

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

*Advance our two lead kinase inhibitors.* Kinase inhibitors have significantly improved the care of many cancer patients and represent a commercially successful category of targeted cancer therapies with sales of over \$29.1 billion in 2011, according to BCC Research. We have two internally discovered novel kinase inhibitors in development: MGCD265 and MGCD516. These product candidates target pathways of high scientific interest, including Met, Axl, TRK, RET, DDR, EphRs and VEGFRs, and are believed to be important in the regulation of tumor growth. We plan to initiate a Phase 2 clinical trial for MGCD265 and a Phase 1 clinical trial for MGCD516 in 2014.



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*Advance mocetinostat, our later-stage product candidate.* HDAC inhibitors have been shown to be effective in treating hematologic malignancies, as evidenced by the approval of Istodax and Zolinza. We have completed 13 clinical trials in 437 patients which have shown promising signs of activity of mocetinostat in MDS and other hematologic malignancies. We believe that the combination of the epigenetic mechanisms of mocetinostat and Vidaza may be effective in treating MDS. Subject to successful completion of our planned dose confirmation trial, we are planning to initiate a Phase 3 registration trial of mocetinostat in 2014 under an SPA to be agreed upon with the FDA for the first line treatment of patients with MDS in combination with Vidaza.

*Leverage partnerships to develop our product candidates.* We plan to collaborate with third parties and partner certain rights to our product candidates as a means to accelerate their broader clinical development and maximize their therapeutic and market potential. We plan to retain certain key development and commercialization rights in our partnerships. We believe that retaining this strategic flexibility will enable us to maximize shareholder value.

#### **Product Candidates**

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

PRODUCT CANDIDATE MGCD265	INDICATION Solid Tumors	<b>TARGETS</b> Met, Axl, VEGFRs	COMMERCIAL RIGHTS Mirati: Global	DEVELOPMENT AND ANTICIPATED MILESTONES
				Initiate expansion cohorts Q1 2014
MGCD516	Solid Tumors	RET, TRK, DDR,	Mirati: Global	Initiate Phase 2 Q4 2014
		EphRs, Met, Axl, VEGFRs		Planned IND submission and initiate Phase 1 1H 2014
				Initiate expansion cohorts Q4 2014
Mocetinostat	MDS	HDACs 1, 2, 3, 11	Taiho: Certain Asian Territories Mirati: All Other Territories	Initiate dose confirmation trial Q4 2013
				Obtain SPA for Phase 3 1H 2014
Our Targeted Kinase Programs				Initiate Phase 3 2H 2014

**STAGE OF** 

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

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

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

#### 2012 Worldwide Retail Sales Figures of Selected Small Molecule Kinase Inhibitors

Brand Name	Sales	/orldwide s(1) (in lions)
Gleevec	\$	4,675
Tarceva	\$	1,401
Sutent	\$	1,236
Nexavar	\$	1,044(3)
Sprycel	\$	1,019
Tykerb	\$	380
Zelboraf <sup>(2)</sup>	\$	249
Xalkori <sup>(2)</sup>	\$	123

(1)

Source: Thomson Pharma.

#### (2)

Launched in 2011.

#### (3)

792 euro converted into U.S. dollars based upon a published exchange rate of 0.7585 euro per U.S. dollar at December 31, 2012.

Our kinase inhibitor programs in clinical development include MGCD265 and MGCD516, which are multi-targeted kinase inhibitors with distinct target profiles. These new molecular entities are in development for the treatment of patients with NSCLC and other solid tumors. We own all global rights to MGCD265 and MGCD516.

#### MGCD265 A Multi-targeted Kinase Inhibitor for Solid Tumors

#### Overview

MGCD265 is an orally-bioavailable, potent, small molecule multi-targeted kinase inhibitor of Met, Axl and VEGFRs. MGCD265 is in development for the treatment of solid tumors, with an initial focus on NSCLC and HNSCC. We have conducted single agent and combination dose escalation trials in 252 patients, with acceptable tolerability and signs of clinical efficacy in patients with advanced solid tumors who have failed standard therapies. Our preclinical studies, in a variety of in vivo tumor models, have suggested that MGCD265 has relatively low toxicity and appears more potent than some of the leading approved kinase inhibitors, including Nexavar, Sutent and Xalkori. We have developed new formulations of MGCD265 designed to increase plasma exposure, improve the degree of target inhibition and increase the likelihood of seeing single agent clinical activity. Assuming one or more of the new formulations achieve sufficient patient exposure in ongoing studies, we intend to select one of the new formulations for introduction into ongoing dose escalation trials with the goal of identifying the MTD by early 2014. Following identification of the MTD, we plan to initiate dose expansion cohorts in patients selected for certain biomarkers.

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Our development strategy for MGCD265 is based on our understanding of the compound's target inhibition profile and, accordingly, our initial focus for this program will include NSCLC and HNSCC. Met and Axl are both over expressed in NSCLC and HNSCC, providing opportunities for targeted patient selection. In addition, we may target patients with certain mutations of Met and Axl that result in oncogenic activation of these targets and may be drivers of tumor growth.

The National Cancer Institute, or NCI, estimates that in 2013, approximately 228,200 patients in the United States will be diagnosed with lung cancer and 159,500 will die due to the disease. Approximately 85% of lung cancers are NSCLCs. Both Met and Axl are over-expressed in NSCLC tumors. Based on published literature, we believe Met to be over-expressed in 40% to 50% of NSCLC tumors, and Axl in over 40%. In addition, the potentially oncogenic mutations of Met and Axl that we are targeting may exist in up to 8% of NSCLC cases. In the United States, it is estimated that there will be approximately 41,400 new cases of head and neck cancer diagnosed and 7,900 deaths in 2013. Approximately 90% of head and neck cancers are HNSCC, and Met is overexpressed in 55% to 85% of HNSCC cases.

#### MGCD265 Market Overview

Although many tumor types may respond to treatment with MGCD265, NSCLC, HNSCC, hepatocellular carcinoma, or HCC, renal cell carcinoma, or RCC, and gastric cancers are of particular relevance to demonstrate the clinical activity of MGCD265. The selection of these indications is based on the expression or over-expression of markers such as Axl, Met and VEGFR. Key features of these markets are shown in the table below.

#### **Estimated Market Size of Certain Cancer Therapies**

<b>Indication</b> Lung Cancer	Supporting Rationale Over-expression of Axl, Met and VEGFR	Precedents for Targeted Therapy Met inhibitor onartuzumab (MetMab) (Phase 2) (including patient selection)	Estimated Market Size (United States, Europe and Japan) \$4.6B in 2011(1) \$5.9B projected in 2021(1)
Head & Neck Cancer	Over-expression of Axl and Met	EGFR inhibitor cetuximab	\$700M in 2011(1)
Renal Cell Carcinoma	Over-expression of Axl, Met and VEGFR	VEGFR / RTK inhibitors: sunitinib, sorafenib, axitinib, bevacizumab/IFN	\$1.6B in 2011(1) \$2.0B projected in 2021(1)
Liver Cancer	Over-expression of Axl, Met and VEGFR	Met inhibitor tivantinib (Phase 2) (including patient selection) RTK inhibitor sorafenib (Phase 3)	\$380M in 2009(3) \$2.0B projected in 2015(2)(3)
Gastric Cancer	Over-expression of Axl, Met and VEGFR	HGF inhibitor rilotumumab (Phase 2) (including patient selection)	\$1.1B in 2011(1) \$2.3B projected in 2021(1)

<sup>(1)</sup> 

Source: Decision Resources, 2012.

(2)

Source: Global Industry Analysts Inc. 2010, Global Data 2010.

(3)

Worldwide market size.

Background

MGCD265 is a small molecule, multi-targeted kinase inhibitor that potently inhibits Axl, Met and VEGFR 1, 2 and 3. These targets have been shown to play key roles in tumor development, tumor survival, tumor escape and blood vessel formation, or angiogenesis. MGCD265 is highly specific for these five targets and shows little to no activity against a panel of over 400 other RTKs. We believe this profile provides the following potential advantages for MGCD265:

therapeutic action against a novel target (Axl);

high specificity reduces the risk of side effects from off-target activity;

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an opportunity to identify patients that express specific markers allowing a predictive and tailored therapeutic strategy using companion diagnostics; and

an opportunity to identify patients whose tumors express genetic alterations that may be drivers of tumor growth and the inhibition of which may demonstrate single agent clinical activity of MGCD265.

Axl is an RTK which has been shown to correlate with clinical-stage and lymph node status in NSCLC. Recent data has shown that Axl is involved in the mechanism of resistance to EGFR inhibitors such as Tarceva. Axl is expressed in other tumor types and may be a significant driver in RCC, ovarian, pancreatic and other tumors.

The Met receptor is a protein that is found on the cell's surface that, when not properly regulated, plays a key role in the growth, survival and metastasis of various types of cancers. The Met target has generated significant scientific and pharmaceutical interest because of its direct involvement in tumor cell survival and angiogenesis. Met expression is elevated in several major tumor types including NSCLC, gastric cancer, RCC and HCC and is associated with poor prognosis. Met activation may also be associated with resistance to EGFR inhibitors such as Tarceva and Iressa and resistance to VEGFR inhibitors such as Sutent. In tumors with Met over-expression, persistent activation of EGFR-dependent signals may be sustained constituting an escape mechanism leading to EGFR-inhibitor resistance. Inhibition of Met appears to block the Met-driven escape mechanism used by tumor cells when treated with other targeted cancer therapies. Similarly, VEGFR resistance may be overcome by inhibiting Met.

#### MGCD265 Preclinical Development

Our preclinical studies, in a variety of in vivo tumor models, have suggested that MGCD265 has relatively low toxicity and appears more potent than some of the leading kinase inhibitors which have recently been approved or are in clinical trials, including Nexavar, Sutent and Xalkori.

In preclinical studies, MGCD265 has demonstrated single agent activity as indicated in the figures below.

Anti-tumor Activity of MGCD265 Compared with Sutent in a Met/HGF-Positive Glioblastoma Model

U87MG tumor cells were injected subcutaneously in immunocompromised mice. When tumor volume reached 50 mm3 mice were treated with MGCD265 or Sutent at the designated dose level or vehicle for 12 days. Tumor volume was measured at designated time points

utilizing Vernier calipers.

Potent Cytoreductive Activity of MGCD265 in a Met Amplified Gastric Cancer Model

Met amplification positive MKN45 tumor cells (5 X106) were implanted subcutaneously in immunocompromised mice. When tumor volume reached 450 mm3 mice were treated with MGCD265 at the designated dose level or vehicle for 16 days. Tumor volume was measured at designated time points utilizing Vernier calipers.

#### MGCD265 Clinical Trials

Multiple Phase 1 clinical trials have been conducted with MGCD265 showing evidence of clinical activity as monotherapy as well as in combination studies. While MGCD265 showed some efficacy and selectively inhibited Met and Axl, it did not reach optimal plasma concentrations or sufficiently inhibit the targets. We are developing a new formulation of MGCD265 designed to increase plasma exposure, improve the degree of target inhibition and increase the likelihood of seeing single-agent clinical activity.

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

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

This Phase 1, open-label, dose escalating clinical trial in patients with advanced solid tumors is evaluating MGCD265, administered orally every day in repeated 21-day cycles. Data is available for 79 patients who were treated with MGCD265 at doses escalating from 24 mg/m<sup>2</sup> QD to a flat dose of 600 mg TID. Nine patients achieved stable disease for more than four months and up to nine months. One of these patients, who had squamous cell cancer, experienced a partial response after ten cycles of treatment based on one target axillary lesion. The non-target bone lesions remained stable. To date the safety profile continues to be favorable in this ongoing Phase 1 program. The most frequent treatment-related adverse events, observed in greater than 10% of patients, or grade 3 adverse events occurring in more than one patient, are summarized in the table below.

#### Adverse Events Observed in MGCD265 Monotherapy 265-101 (n=79)

### Most frequent treatment-related adverse

events				
	(>10%, all grades)	Grade 3 adverse events occurring in > 1 patient		
Diarrhea		52%	Diarrhea	n=3 (DLT n=1)
Fatigue		30%	Fatigue	n=3 (DLT n=1)
Nausea		33%	Lipase elevation	n=2 (DLT n=1)
Anorexia		25%	Alk phosphatase elevation	n=2
Vomiting		20%		

We expect to continue enrollment in this trial upon completion of ongoing MGCD265 formulation work.

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

In December 2011, we completed an open label Phase 1 clinical trial with dose-escalation of MGCD265 in patients with advanced solid tumors. We enrolled 47 patients with advanced solid tumors. Four patients (papillary renal cell, sarcomatoid bladder, neuroendocrine, and head and neck cancers) had prolonged stable disease with durations ranging from 4 to 12.9 months. The patient with sarcomatoid bladder cancer was stable for 7.5 months and exhibited decreases in Met and phospho-Met protein expression, as well as a change in intact vascular structures, in a post-treatment biopsy sample. The most frequent treatment-related adverse events, occurring in greater than 10% of patients, included diarrhea (30%), nausea (26%), and fatigue (26%). Most of these adverse events were reported as grade 1 or 2 in severity. The observed dose limiting toxicities, or DLTs, were grade 3 mood alteration (n=1) and grade 3 fatigue in the same patient and grade 3 hemoptysis (n=1) all at the dose of 170 mg/m<sup>2</sup> BID (n=6). An additional grade 3 adverse event of increased lipase was also reported at a dose of 192 mg/m<sup>2</sup> BID (n=1).

#### Phase 1/2 Clinical Trial Evaluating MGCD265 in Combination with Taxotere or Tarceva (Ongoing)

This dose-escalating Phase 2 clinical trial is evaluating MGCD265 in combination with Taxotere or Tarceva. Data is available for 124 patients treated with MGCD265 at doses of up to 700 mg BID taken with meals and administered in combination with full dose Tarceva or Taxotere. Overall, the treatment was well tolerated and the adverse events observed are generally those associated with Taxotere or Tarceva treatment.

As of the last data review in July 2013, 56 patients have been treated in the MGCD265-plus-Taxotere arm of our combination trial. Taxotere was started at a dose level of 50 mg/m<sup>2</sup> (first dose level) and then escalated to 75 mg/m<sup>2</sup> in combination with MGCD265.

Data is available for 56 patients treated with MGCD265 at doses of up to 700 mg BID taken with meals and administered in combination with Taxotere. Stable disease for 6 to 18 months was observed in six patients: NSCLC (n=4), ovarian and pancreatic cancer (n=1 each). Objective partial responses were observed in two out of sixteen patients with NSCLC, one out of four patients with prostate cancer, one out of two patients with head and neck cancer and the only patient with endometrial cancer.

The most common treatment-related non-hematologic adverse events observed to date have been constitutional or gastro-intestinal related and are summarized in the table below. Expected Taxotere associated adverse events of anemia (n=3, grade 3), leucopenia (n=5, grade 3-4) and neutropenia (n=31, grade 3 and 4) and two cases of febrile neutropenia have also been observed. Grade 3 or higher adverse events occurring in more than one patient are summarized in the table below. In addition, one patient was reported to have a pulmonary embolism (grade 4) that was an incidental finding on CT scan. Another patient who had advanced NSCLC and was oxygen dependent at baseline, was diagnosed with fatal pneumonitis (inflammation of the lung tissue) (grade 5) in the context of worsening pleural effusion and increasing parenchymal consolidation. Additional cycle 1 DLTs reported in this study include grade 3 diarrhea (n=1), grade 3 lipase and grade 3 fatigue (n=1), grade 3 lipase (n=1), grade 3 AST (n=2), and

grade 4 lipase and grade 2 pancreatitis (n=1) in a patient with grade 3 lipase at baseline consistent with chronic pancreatitis.

#### Adverse Events Observed in MGCD265 Combination Therapy Combination Therapy 265-103 with Taxotere (n=56)

#### Most frequent treatment-related adverse

events (>10%, all grades)		Grade 3 adverse events or higher occurring in > 1 patient		
Fatigue	50%	Neutropenia	n=31	
Alopecia	46%	Leucopenia	n=5	
Diarrhea	41%	Diarrhea	n=3 (DLT n=1)	
Nausea	30%	Elevated lipase	n=3 (DLT n=3)	
Anorexia	25%	Hypophosphatemia	n=2	
Vomiting	18%			
Mucosal inflammation	16%			
Taste disturbance	16%			
Constipation	14%			
Myalgia	11%			
Rash	11%			

As of the last data review in July 2013, 68 patients have been treated in the MGCD265-plus-Tarceva arm of our combination trial. Tarceva was started at a dose level of 100 mg (first dose level) and then escalated to 150 mg in combination with MGCD265.

Data is available for 68 patients treated with MGCD265 at doses of up to 700 mg BID taken with meals and administered in combination with Tarceva. Seven patients with a variety of tumors have experienced stable disease for six months or more. One NSCLC patient had a partial response (also positive for EGFR activating mutation). Three out of nine patients with gastroesophageal cancer remained on study for approximately 11 to 34 months. Overall, the combination of MGCD265 with Tarceva has been well tolerated and the most common treatment-related adverse events are consistent with known Tarceva toxicity and include skin-cutaneous or gastro-intestinal related events. The most frequent treatment-related adverse events are summarized in the table below. No grade 4 or grade 5 toxicities have been reported in the Tarceva combination study.

#### Adverse Events Observed in MGCD265 Combination Therapy with Tarceva Combination Therapy 265-103 with Tarceva (n=68)

events			Grade 3 adverse events	
(>10%, all grades)			occurring in > 1 patient	,
Diarrhea	81%	Diarrhea		n=12 (DLT n=5)
Fatigue	46%	Hypokalemia		n=3
Rash	35%	Hypophosphatemia		n=2
Anorexia	27%			
Nausea	22%			
Dermatitis acneiform	21%			
Dry skin	15%			
Hypokalemia	10%			
Taste disturbance	10%			

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#### Phase 1 Clinical Trial Evaluating the Pharmacokinetics of MGCD265 in Healthy Volunteers in a Fed versus Fasted State (Complete)

In 2012, a Phase 1 study in healthy volunteers (n=14) was conducted to compare the pharmacokinetics of a single 100 mg dose of MGCD265 under fed conditions versus those after a 10-hour overnight fast. Safety was evaluated in all subjects for seven days after each single dose. On average, the fed condition was associated with an approximately three-fold increase in exposure. All treatment-related adverse events were mild except for one patient who reported moderate diarrhea when dosed under fasting conditions. The study results indicated that exposures could significantly improve up to three-fold in the fed subjects and provided support for the formulation improvement work that was undertaken earlier this year.

#### MGCD265 Developmental Initiatives and Objectives

Since January 2013, we have developed new formulations of MGCD265 designed to increase plasma exposure, improve the degree of target inhibition and increase the likelihood of seeing single-agent clinical activity. Assuming one or more of the new formulations achieve sufficient patient exposure in ongoing studies, we intend to select one of the new formulations for introduction into ongoing dose escalation trials with the goal of identifying the MTD by early 2014. After the MTD is identified, we plan to initiate dose expansion cohorts in patients selected for Met and/or Axl over-expression as well as a cohort of patients that have genetic mutations of Met or Axl that we believe are drivers of tumor growth. Our initial focus for this program will include both NSCLC and HNSCC. Because the trial is open-label, we may see evidence of clinical activity from the expansion cohorts by mid-2014.

We believe that by selecting patients with over-expression of Met and/or Axl as well as patients with genetic mutations associated with pathways that are critical to tumor growth that are potently inhibited by MGCD265 may increase the likelihood of seeing clinical activity earlier in clinical development. We are currently using commercially available diagnostic assays as well as assays developed internally for early clinical uses. We are developing companion diagnostics in collaboration with third parties that we plan to use for later stage registration trials and commercialization, if approved.

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

MGCD516 is an orally-bioavailable, potent, small molecule multi-targeted kinase inhibitor of RET, TRK, DDR and EphRs, as well as Met, Axl and VEGFRs, in development for the treatment of solid tumors. We plan to focus on solid tumors expressing RET, TRK and DDR, initially in NSCLC, and we plan to evaluate other tumor types where the profile of MGCD516 would suggest activity. MGCD516 is in advanced preclinical development. We plan to file an IND with the FDA, and initiate a Phase 1 clinical trial of this product candidate in the first half of 2014, and identify the MTD and initiate expansion cohorts in patients selected for certain biomarkers by the end of 2014.

MGCD516 has shown potent inhibition in vitro of cell proliferation, cell motility and angiogenesis. In preclinical animal studies, MGCD516 shows good oral bioavailability in mice, rats and dogs, and anti-tumor activities in multiple human xenograft tumor models in mice.

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In preclinical lung cancer studies, MGCD516 demonstrated better tumor volume reduction activity than Nexavar as shown in the figure below.

Anti-tumor Activity of MGCD516 Compared with Nexavar in the A549 Lung Cancer Model

A549 tumor cells were injected subcutaneously in immunocompromised mice. When tumor volume reached 50 mm3 mice were treated with MGCD516 or Nexavar at the designated dose level or vehicle for 13 days. Tumor volume was measured at designated time points utilizing Vernier calipers.

MGCD516 also demonstrated significant activity in a RET fusion lung cancer preclinical model shown in the figure below.

Potent Cytoreductive Activity of MGCD516 in a RET Fusion-Positive Lung Cancer Model KIF5B-RET fusion positive primary tumors were implanted subcutaneously in immunocompromised mice. When tumor volume reached 200 mm3 mice were treated with MGCD516 at the designated dose level or vehicle for 16 days. Tumor volume was measured at designated time points utilizing Vernier calipers.

In addition, MGCD516 demonstrated activity in overcoming VEGFR resistance in a preclinical model treated with Sutent, as shown below.

Cytoreductive Anti-tumor Activity of MGCD516 in a Sutent-Resistant Tumor Model

Primary tumors were implanted subcutaneously in immunocompromised mice. When tumor volume reached 250 mm3 mice were treated with Sutent for 44 days until they progressed to a volume of 600 mm3. Mice were randomized into two groups and treated with MGCD516 or Sutent at the designated dose level or vehicle for 18 additional days. Tumor volume was measured at designated time points utilizing Vernier calipers.

## Mocetinostat An Oral HDAC Inhibitor for MDS

#### Overview

Mocetinostat is an orally-bioavailable, spectrum-selective HDAC inhibitor for which we plan to conduct a dose confirmation trial starting in the fourth quarter of 2013, with the goal of initiating a Phase 3 clinical trial in the second half of 2014. We have completed 13 clinical trials which enrolled 437 patients with a variety of hematologic malignancies and solid tumors. We intend to seek an SPA from the FDA prior to the initiation of our planned Phase 3 trial. This trial will evaluate mocetinostat for the first line treatment of patients with MDS in combination with Vidaza, an HMA. We believe that mocetinostat has the potential to be the first HDAC inhibitor to market for this indication.

We believe that the epigenetic mechanisms of HDAC inhibitors and HMAs may be complementary in the treatment of MDS. Epigenetics is the regulation of gene expression and resulting cellular phenotypes through mechanisms other than primary DNA sequence alterations. The epigenetic regulation of gene expression involves the regulation of DNA methylation and modification of certain histones via modulation of acetylation or methylation of specific amino acid residues. Epigenetic pathways can become dysregulated during cancer progression through a variety of mechanisms, including the genetic alteration of molecules that participate in DNA methylation and histone modification. These alterations often result in silencing of selected tumor suppressor genes and uncontrolled tumor growth in certain malignancies including MDS and lymphomas. Because the epigenetic regulation of gene expression is controlled by DNA methylation and histone modification, we have focused on the development of mocetinostat for the treatment of MDS in combination with HMAs.

We partnered mocetinostat with Pharmion Corporation (predecessor to Celgene Corporation) in 2006. In 2008, Celgene voluntarily placed the mocetinostat program on clinical hold with the FDA following an

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observation of pericarditis in clinical trials. Celgene subsequently terminated the collaboration in January 2009 and all rights to the mocetinostat program reverted to us. Following the termination and based upon a review of the safety data and discussion with the FDA, the clinical hold was removed in 2009. However, no further development was conducted by us under our prior management team. When our current management team joined us in late 2012, we began a detailed portfolio review and subsequently determined that further development of mocetinostat was warranted.

#### Mocetinostat Market Overview

The potential of HDAC inhibitors for the treatment of hematological malignancies has already been validated by the approval of two HDAC inhibitors, Zolinza and Istodax, for the treatment of T-cell lymphoma. Our clinical studies of mocetinostat indicate that this agent may have promising activity in MDS and other hematological malignancies such as Hodgkin's lymphoma, or HL, and non-Hodgkin's lymphomas, or NHL, including diffuse large B-cell lymphoma, or DLBCL, and follicular lymphoma, or FL.

Our primary focus for mocetinostat is on the first line treatment of patients with MDS. MDS consists of a group of heterogeneous, clonal hematopoietic stem cell disorders that are characterized by abnormal bone marrow and blood cell development. According to NCI, MDS will be diagnosed in more than 10,000 people in the United States in 2013. Utilizing Surveillance Epidemiology and End Results data from NCI, Decision Resources estimates the prevalence of MDS to be over 52,000 patients in the United States and over 49,000 patients in the European Union.

MDS is a complex and heterogeneous disease, divided into patient subgroups with differing therapy objectives. The International Prognostic Scoring System, or IPSS, for MDS was developed to assess patient prognosis and guide the course of treatment of MDS, and utilizes clinical variables such as bone marrow blast percentage, number of peripheral blood cytopenias and cytogenic risk group to categorize MDS patients and provide prognostic expectations. Approximately one-quarter of MDS patients are classified as high-risk (Intermediate-2 or high IPSS risk category). Prognosis for these high-risk MDS patients is generally poor and there is a significant medical need for therapeutic regimens that will improve clinical outcomes.

The standard of care, according to the National Comprehensive Cancer Network, for first line therapy for Intermediate-2 and high-risk patients is treatment with HMAs. The HMAs Vidaza and Dacogen are approved as first line agents for the treatment of high-risk MDS patients in the United States, and 2012 U.S. sales were approximately \$327 million and \$240 million, respectively. Although these therapies represent the standard of care for the treatment of high-risk MDS patients, only a minority of patients achieve an objective response. Almost all patients who initially respond to therapy eventually relapse, and the survival time of MDS patients who have failed HMAs is less than six months. Allogenic hematopoietic stem cell transplantation, or HSCT, is the only potential curative treatment for MDS; however, its use is restricted to a relatively small number of eligible patients and requires an appropriate donor.

NHL (including the aggressive DLBCL and FL) is the most common form of blood cancer. NCI estimates that 70,000 patients will be diagnosed with NHL in the United States in 2013 and that the incidence has grown annually over the past ten years. The prevalence of NHL in the United States is 509,000. Aggressive NHL is treated with rituximab (anti-CD20) plus chemotherapy, which is effective in about 67% of cases, but relapsed or refractory aggressive NHL has a poor outlook with limited therapeutic options.

NCI estimates that 9,300 patients will be diagnosed with HL in the United States in 2013 and that the prevalence of HL in the United States is 182,000. Treatments of HL typically include radiation, chemotherapy and HSCT. Chemotherapy followed by consolidation radiation therapy is the most effective treatment for early-stage HL. Current approaches seek to balance efficacy against the risk of long-term complications such as cardiac disease and other types of cancer. Patients with refractory HL currently have few therapeutic options such as high dose chemotherapy followed by stem cell transplant or brentuximab vedotin (anti-CD30 antibody conjugated to cytotoxin).

We believe that a significant unmet medical need remains for effective treatment that increases the response rates in patients with Intermediate-2 and high-risk MDS, HL and NHL.

#### Mocetinostat Background

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

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

#### Mocetinostat Clinical Development

Our IND for mocetinostat was submitted in December 2003 and became effective in January 2004. To date, we have evaluated mocetinostat as a monotherapy and in combination with other anticancer agents in 437 patients in Phase 1 and Phase 2 clinical trials with various malignancies, including MDS, HL, NHL (including DLBCL or FL), AML, chronic lymphocytic leukemia and chronic myelogenous leukemia, as well as advanced solid tumors. Through these trials, the safety and tolerability of mocetinostat as a single agent and in combination has been well characterized. The clinical trials showed activity as a single agent in HL and NHL and in combination with Vidaza in MDS and AML.

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

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

## CLINICAL TRIALS EVALUATING MOCETINOSTAT

## MDS

In late 2012 and early 2013, our new management team reviewed the data from our prior clinical trials of mocetinostat. As a result, our management team concluded that the combination of mocetinostat and Vidaza demonstrated clinically meaningful responses in MDS patients and demonstrated an improvement over published responses of Vidaza alone. The objective response rate of Vidaza, as shown in its product label, is 15.7%, with 5.6% of patients achieving a complete response, or CR, compared to CR's in 11% of the patients in the mocetinostat data as set forth in the table below. A complete response generally refers to the disappearance of all signs of cancer in response to treatment, while a partial response generally refers to a decrease in the size of the tumor or in the extent of cancer in the body.

In an open-label, Phase 1/2 trial of patients with MDS or AML that was conducted starting in 2006, we evaluated the activity of mocetinostat in combination with Vidaza in patients with MDS. A total of 66 subjects were enrolled, including 28 patients with MDS as assessed by independent analysis. Patients with MDS were treated with mocetinostat at starting doses of 35 to 135 mg three times weekly, with most patients starting at 90 mg, and continued treatment until disease progression or prohibitive toxicity. Among the 28 patients with MDS, the ORR (CR+CRi+HI) was 61% (17 of 28), and the disease control rate (CR+CRi+HI+SD) was 93% (26 of 28) in an independent assessment. A summary of this data is set forth in the table below.

## MOCETINOSTAT COMBINED WITH VIDAZA PHASE 2 CLINCIAL DATA

MDS Best Response Rates (n=28)	n (%)
CR+CRi	14 (50)
CR	3 (11)
CRi	11 (39)
HI	3 (11)
SD	9 (32)
ORR (CR + CRi +HI)	17 (61)
Disease Control (CR + CRi + HI + SD)	26 (93)

CR = complete response; CRi = complete marrow response but without normalization of peripheral counts; HI = hematologic improvement; SD = stable disease; ORR = objective response rate.

#### Mocetinostat Safety

In the mocetinostat plus Vidaza study, the most commonly reported adverse events are set forth in the tables below. These adverse events are generally consistent with those seen in MDS and AML patients treated with this class of agent.

Most Common Treatment-related Adverse Events (All Grades) Mocetinostat with Vidaza, MDS & AML Patients		Treatment-related Adverse Events (Grades 3 and 4) Mocetinostat with Vidaza, MDS & AML Patients				
EVENT	Patients, n(%)	EVENT	<b>Grade 3(1)</b>	Grade 4		
Nausea	44 (67)	Fatigue	15 (23)	0 (0)		
Diarrhea	43 (65)	Nausea	14 (22)	0 (0)		
Fatigue	32 (49)	Diarrhea	11 (17)	1 (2)		
Anorexia	30 (46)	Vomiting	9 (14)	0 (0)		
Asthenia	22 (33)	Anemia	6 (9)	1 (2)		
Weight loss	16 (24)	Anorexia	6 (9)	0 (0)		
Thrombocytopenia	13 (20)	Dehydration	5 (8)	0 (0)		
Anemia	10 (15)	Asthenia	4 (6)	0 (0)		
Hypokalemia	9 (14)	Thrombocytopenia	3 (5)	6 (9)		
Constipation	8 (12)	Leukopenia	2 (3)	2 (3)		
Dysgeusia	8 (12)	Neutropenia	1 (2)	4 (6)		
Dehydration	7 (11)	_				
Dizziness	7 (11)					

(1)

Excludes Grade 3 adverse events with an incidence less than 5%.

#### Pericarditis Finding and Clinical Hold

In July 2008 Celgene instituted a voluntary clinical hold to new patient enrollment for mocetinostat, which was accepted by the FDA in August 2008. The voluntary clinical hold was put in place in response to an observation of pericarditis and pericardial effusion (inflammation of the pericardium, the fibrous sac surrounding the heart, and accumulation of fluid around the heart).

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We provided the FDA with an integrated analysis of pericardial events identified in mocetinostat clinical studies. A causal association of mocetinostat with pericardial events was not established since the observed events could be related to the patient population and their prior therapy. Of the 437 patients treated with mocetinostat, there have been a total of 19 patients (4.3%) who had serious adverse events, or SAEs, where a pericardial adverse event was mentioned, and a total of 45 patients (10.3%) who had pericardial findings, which included the 19 SAE findings as well as 27 incidental findings identified through reviews of on-study CT scans, database searches and prospective echocardiogram monitoring, which did not have significant clinical sequellae. Only one pericardial SAE occurred among the 28 patients (3.6%) with MDS in the Phase 1/2 clinical trial. Based on literature reviews and other investigator-driven reviews, the rate of pericardial findings is approximately 10% of cancer patients, but rates have been reported to vary from 3% to approximately 40% for patients with advanced cancers who may have received multiple previous anticancer therapies. However, the potential exists for a relationship with treatment, and the possibility of mocetinostat being a contributing factor to the occurrence of pericardial events has not been excluded. We agreed with the FDA that the best way to assess the risk of pericarditis is in a sufficiently large randomized study of safety and efficacy.

Our complete response to the voluntary clinical hold was accepted by the FDA and the hold was lifted in September 2009. Our response included specific guidance for identifying patients at potential risk for, and guidance to manage patients who develop, pericarditis or pericardial effusions. As a result, new patient enrollment in mocetinostat clinical trials will include both the exclusion of patients who are diagnosed with cardiac abnormalities prior to starting mocetinostat therapy (i.e. myocardial infarction, congestive heart failure and pericardial disease) and patient monitoring by electrocardiogram and echocardiography at baseline and while on study. These diagnostic tests are non-invasive and relatively common procedures. The three patients with lymphoma who were enrolled after the voluntary clinical hold was lifted did not show signs of pericarditis or pericardial effusions.

#### Lymphoma

We tested the safety and efficacy of mocetinostat in patients with relapsed HL in a trial starting in 2006. Two doses were assessed (85 mg and 110 mg three times weekly), and patients were treated until disease progression or prohibitive toxicity. A total of 51 patients were enrolled. On the basis of intent-to-treat analysis, the disease control rate was 35% (8 of 23 patients) in the 110 mg group and 25% (7 of 28) in the 85 mg group. A total of 12 patients discontinued treatment because of adverse events, nine in the 85 mg cohort and three in the 110 mg cohort. The most frequent treatment-related grade 3 and 4 adverse events were neutropenia, fatigue and pneumonia. Four patients in the 110 mg cohort died during the study.

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

#### Mocetinostat Developmental Plans

Subject to successful completion of our planned dose confirmation trial, we are planning to initiate a Phase 3 registration trial for mocetinostat in the second half of 2014. The proposed randomized trial is intended to support regulatory approval of mocetinostat in combination with Vidaza for the treatment of patients with Intermediate or High-Risk MDS who have not previously received Vidaza, and for whom Vidaza is indicated.

In advance of the Phase 3 trial, we plan to conduct a dose confirmation trial to confirm the planned Phase 3 dose and to obtain further clinical data and test safety monitoring protocols. The study is expected to be initiated in the fourth quarter of 2013 and to include approximately 30 patients, with 10 patients

treated with mocetinostat at the 70 mg dose level and 20 patients treated at the 90 mg dose level, each in combination with Vidaza, in a single arm open-label study. The current proposed dose of mocetinostat for this Phase 3 clinical trial is 90 mg.

The proposed registration trial is expected to be a 1:1 randomized study comparing mocetinostat plus Vidaza with Vidaza alone in HMA naïve subjects who have been diagnosed with MDS and that have met criteria for the risk groups of Intermediate or high-risk according to IPSS. Although we have guidance from the FDA on certain key points, we intend to seek an SPA from the FDA on the design of the Phase 3 clinical trial. We intend to propose to the FDA an adaptive study design and intend to discuss both ORR and OS as potential endpoints for the basis for approval. While the detailed clinical and statistical plan are still under discussion and evaluation, the size range for the trial is currently estimated to be between 250 and 500.

We anticipate that the trial will include eligibility criteria to exclude patients with pre-existing pericardial effusion, on-study monitoring including echocardiograms and electrocardiograms in both treatment arms, and data monitoring safety committee oversight for potential adverse events including those specifically related to pericardial events.

We are also evaluating potential future development of mocetinostat in patients with NHL and HL.

#### **Strategic Alliances and Commercial Agreements**

## Collaboration with Taiho

In October 2003, we entered into a license and research and development collaboration agreement with Taiho, a leading Japanese specialty oncology company, for mocetinostat and our small molecule HDAC inhibitor program for oncology for Japan, South Korea, Taiwan and China, or collectively the Taiho Territory. Under the terms of the agreement, we received an up-front license fee, equity investment and a contract research payment of \$3.8 million. In addition, we may receive milestone payments based on successful development, regulatory approval, and commercialization of an HDAC oncology product totaling up to \$16.2 million. We may also receive royalty payments in connection with commercial sales of HDAC oncology products as a percentage of annual net sales, which percentage is in the mid-single digit to mid-teen percent range, depending upon the total dollar amount of annual net sales. Such royalties may be reduced, subject to a mid-single digit floor, by (i) credits against recoupable development costs paid by Taiho to us and/or (ii) reduction by a percentage in the range of 20-30% in the event a generic competitor is introduced in a particular market, other than in China. Taiho provided us with contract research payments for scientists for two years at \$2.0 million per year as well as funding for contract preclinical and contract clinical development costs in North America for mocetinostat, which totaled, in the aggregate, \$5.4 million. In total, we have received \$15.0 million from Taiho under the agreement, including a \$1.5 million milestone payment relating to the start of the first Phase 2 trial with mocetinostat. However, upon the execution of our agreement with Celgene in 2008, Taiho's funding obligations for clinical trials in North America ceased. In addition, Taiho's collaboration entailed in-kind support in their research laboratories in order to select a next generation compound, and in some cases, will support a portion of preclinical development costs in North America. Currently, there are no efforts by either (i) Taiho to further advance mocetinostat in the Taiho Territory or (ii) Taiho or us to further advance other small molecule HDAC inhibitors that would be covered by this agreement. However, Taiho has retained rights in the Taiho Territory to certain sirtuin inhibitors for cancer. The term of the agreement will, on a country-by-country basis, continue until expiration of the last to expire issued patent, or ten years after the first commercial sale in Japan. Additionally, Taiho has a unilateral right to terminate the agreement for any reason with 30 days written notice, and we have a unilateral right to terminate the agreement if Taiho fails to make an undisputed payment. An arbitrator may terminate the agreement for a breach of obligations if such breach has remained uncured for 90 days. As long as the agreement continues, we are obligated to use reasonable efforts to contract with Taiho for our supply of the active bulk compounds for the sale of mocetinostat outside of the Taiho Territory. In the event the parties wish to collaborate on the development of another HDAC inhibitor covered by this agreement or sirtuin inhibitor retained by Taiho, Taiho would be obligated to



contribute to preclinical and clinical costs of such a compound. Such a compound would also be subject to potential development milestones and royalties. We are in preliminary discussions with Taiho to consider whether any amendments to the agreement should be made based upon our development plans for mocetinostat and their rights under the agreement.

#### Collaboration with Otsuka

In March 2008, we entered into a worldwide research collaboration and license agreement with Otsuka, a global Japanese pharmaceutical company, for the development of novel, small molecule, kinase inhibitors for local delivery and treatment of ocular diseases, excluding cancer. We were responsible for the design, characterization and initial screening of kinase inhibitors and control over determining which compounds to synthesize. Otsuka was responsible for funding efficacy and toxicity studies, as well as preclinical and clinical development of compounds. Otsuka is also responsible for the global commercialization of any resulting product. Under the terms of the agreement, we received an up-front license fee of \$2.0 million. We may receive additional payments based on successful development, regulatory, commercialization and sales milestones that could total up to \$50.5 million. We may also receive royalty payments in connection with commercial sales of licensed products under the agreement as a percentage of annual net sales, which percentage is in the mid-single digit to mid-teen percent range, depending upon the total dollar amount of annual net sales, subject to reduction by a percentage in the range of 40-50% in the event a generic competitor is introduced in a given market or intellectual property protection in a particular market does not exist or expires in a given market. We may receive aggregate milestone payments of up to \$50.5 million under this agreement as follows: \$7.5 million relates to development activities, \$22.0 million relates to the completion of regulatory approvals and \$21.0 million relates to the achievement of certain sale goals. Otsuka provided \$1.9 million in research funding for the initial 18 months of the research collaboration, which was extended on three occasions: September, 2009; April 2010 and June 2010. The research component of the agreement ended on June 30, 2011. We received a total of \$4.5 million in research funding from the research component of this agreement. In October 2009, Otsuka made, in connection to the terms of the agreement, a \$1.5 million equity investment in our shares of common stock at a share price of CND\$21.30 (or US\$20.27, as converted), which was a 20% premium over the five-day volume-weighted average closing price at the date of the transaction. On June 30, 2010, the collaboration agreement was amended to, among certain other changes, provide Otsuka the rights to synthesize a limited number of compounds predetermined by us. A lead molecule was selected in June 2011 for further development. The research portion of the collaboration between us and Otsuka concluded on June 30, 2011; however, the term of the agreement will, on a country-by-country basis, continue until expiration of the last to expire issued patent, or if no patent has issued in such country, then 12 years after the first sale of a licensed product by Otsuka. Otsuka has a unilateral right to terminate the agreement for any reason with 90 days written notice and either party may terminate the agreement for a breach of obligations of the other party if such breach has remained uncured for 120 days (or 30 days for a breach of payment). Otsuka is currently advancing the lead compound through late preclinical development.

#### Collaboration with EnVivo

In March 2004, we entered into a proof of concept and option agreement with EnVivo, a private U.S. biotechnology company focusing on the treatment and prevention of certain neurodegenerative diseases, to exploit our HDAC inhibitors in diseases such as Huntington's disease, Parkinson's disease and Alzheimer's disease. In February 2005 we signed an exclusive research, collaboration and license agreement. Over the course of 2005, EnVivo paid us \$0.6 million for research, plus a \$0.5 million license fee, for a total of \$1.1 million. As part of this agreement, EnVivo received a warrant to purchase 1,050 shares of common stock at an exercise price of CND\$214.30 (or US\$203.76, as converted). The warrant expired in March 2007. In February 2008, we exercised our right to opt-out of the program. As a result, we granted EnVivo exclusive rights to our HDAC inhibitors for neurodegenerative diseases and we ceased research and development funding for this program. We are prohibited under the surviving terms of the agreement with EnVivo from developing or commercializing any HDAC products in the field of certain neurodegenerative diseases, including Huntington's disease, Parkinson's disease and Alzheimer's disease. We may receive

royalty payments in an aggregate amount equal to a single digit percentage of net sales of any approved compound and will share in any sublicense income from future partnerships that EnVivo may enter into.

#### **Intellectual Property**

### Patents and Proprietary Technology

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

We typically file for patents in the United States with counterparts in certain countries in Europe and certain key market countries in the rest of the world, thereby covering the major pharmaceutical markets. As of September 30, 2013, we own or co-own 52 U.S. patents and patent applications and their foreign counterparts, including 25 issued U.S. patents as reflected in the following table:

Granted (United States)	Pending (United States)
8	11
10	15
6	1
1	0
25	27
	<b>States)</b> 8 10 6 1

#### Granted and Pending U.S. Patents

#### Kinase (8 granted U.S. patents; 11 pending U.S. patent applications)

As of September 30, 2013, we have eight issued patents and eleven pending patent applications in the United States covering inhibitor compounds, including MGCD265 and MGCD516, and methods of use of these compounds. Of these issued patents, one covers multiple series of kinase inhibitors and protects MGCD265 generically. Another issued patent, which expires no earlier than 2026, protects a selection of compounds including MGCD265, as well as methods of inhibiting VEGF and HGF receptor signaling and methods of treating angiogenesis-mediated cell proliferative disease or inhibiting solid tumor growth. Exclusivity arising from our issued patents for MGCD265 extends to at least 2026, including our patents covering the specific composition of matter of MGCD265 (expires 2026, prior to any legal or regulatory extentions, including any patent term extension, that may be available under the Hatch Waxman Act) and the generic class of compounds to which MGCD265 belongs (expires 2025, prior to legal or regulatory extensions, including any patent four issued patents cover several distinct classes of compounds. Such coverage includes specific claims to MGCD516, generic coverage of the class of compounds to which MGCD516 belongs, as well as patents covering methods of use of such compounds. Exclusivity arising from our patents covering methods of use of such compounds. Exclusivity arising from our patents covering methods of use of such compounds. Exclusivity arising from our patents covering methods of use of such compounds. Exclusivity arising from our patent protection for

MGCD516 extends to at least 2029, prior to legal or regulatory extensions, including any patent term extension that may be available under the Hatch Waxman Act.

Our pending patent applications relating to our kinase inhibitors seek coverage of a broader scope of kinase inhibitors both for oncology and for the treatment of ophthalmic diseases. Methods of use of these inhibitors, such as methods of inhibiting VEGF and HGF receptor signaling, methods of treating angiogenesis-mediated cell proliferative disease or inhibiting solid tumor growth, as well as processes of manufacturing kinase inhibitors such as MGCD265 and synthetic intermediates required for the purpose are also being pursued.

### Hos2 and HDAC Programs (10 granted U.S. patents; 15 pending U.S. patent applications)

Our patent estate for our Hos2 and HDAC programs covers multiple series of HDAC inhibitors, including MGCD290 and mocetinostat. This group of patents includes 10 issued patents and 15 pending patent applications in the United States protecting composition of matter and method of use. One issued patent covers the Hos2 inhibitor MGCD290 both generically and specifically. Exclusivity arising from our patent protection for MGCD290 should extend to at least 2020, and exclusivity arising from our issued patents claiming the combination of MGCD290 with antifungal agents extends to 2026, prior to any legal or regulatory extensions that may be available to us. Exclusivity for mocetinostat extends to 2022 prior to legal or regulatory extensions, including any patent term extension that may be available under the Hatch Waxman Act.

In aggregate, these U.S. patents and patent applications cover the following inventions: novel HDAC inhibitors, including mocetinostat (eight issued patents and nine patent applications), methods of inhibiting HDACs, methods for treating cell proliferative disease or cancer, specific methods for treating colon, lung and pancreatic cancers, methods for treating polyglutamine expansion diseases (such as Huntington's disease) and methods for treating fungal infection. Three applications claim compositions of HDAC/Hos2 inhibitors with antifungal compounds, methods of enhancing the activity of the antifungal compounds with HDAC/Hos2 inhibitors, and methods of treating fungal infection. One pending application also seeks protection of the analogs of MGCD290 as well as prodrugs of HDAC/Hos2 inhibitors and their use, while another pending application claims methods for identifying/screening potentiators of antifungal compounds, the inhibitors of ergosterol biosynthesis. A provisional application is directed to novel HDAC/Hos2 inhibitors and their use.

## Beta-Lactamase (6 granted U.S. patents; 1 pending U.S. patent applications)

For our beta-lactamase inhibitor program, we co-filed two patent applications with Merck and Merck has since returned all rights to these patents to us. In line with our corporate objectives to promote the partnering and development of our lead beta-lactamase inhibitor, MG96077, we are currently supporting the prosecution of only one granted patent (coverage until 2027) that protects this molecule both specifically and generically. The majority of the other patents are in the process of abandonment.

## DNMT Program (1 granted U.S. patent)

In our DNA methyltransferase program, we own one U.S. patent specifically covering MG98. This U.S. patent covers MG98 and methods for inhibiting tumor growth with it. We may abandon this patent in the future as we are no longer pursing this program.

#### **Licensing Agreements**

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

#### Competition

## Competitors in Oncology Small Molecule Kinase Inhibitors

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

The Met kinase inhibitor field has recently generated intense scientific and industry interest. We believe that most of the biotechnology and pharmaceutical companies developing small molecule drugs for cancer

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have significant and active kinase inhibitor programs (including Met programs) that may be competitive with our own and these competitors are described below. Our MGCD265 program is attractively positioned in the pipeline of Met-targeted molecules and is characterized by potential advantages including: a unique kinase spectrum including the emerging RTK target Axl; a lack of activity against over 400 off-target kinases, supporting a favorable safety profile; and excellent tolerability to date with other anti-cancer agents (including chemotherapy), thus optimizing the potential for combination therapy approaches.

Companies with Met inhibitors believed to be in late preclinical or clinical development include, but are not limited to: Amgen Inc., ArQule Inc. and its partners Kyowa Hakko Kirin Pharma Inc. and Daiichi Sankyo Company Limited, Aveo Pharmaceuticals Inc., Bristol-Myers Squibb Company, Exelixis Inc., F. Hoffman-LaRoche Ltd., GlaxoSmithKline PLC, Novartis AG and Pfizer.

Axl is a newly emergent RTK target. However, a small number of RTK inhibitors that are launched in development are believed to inhibit Axl. These include foretinib (in Phase 2 development by Exelixis) and Xalkori.

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

#### Competitors in Oncology Mocetinostat Competitors

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

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

## Competitors in Oncology General Competitors

In addition to companies that have HDAC inhibitors or kinase inhibitors addressing oncology indications, our competition also includes hundreds of private and publicly traded companies that operate in the area of oncology but have therapeutics with different mechanisms of action. The oncology market in general is highly competitive, with over 1,000 molecules currently in clinical development. Other important competitors, in addition to those mentioned above, include, but are not limited to: small and large biotechnology companies, including but not limited to Amgen, Ariad Pharmaceuticals Inc., ArQule, Biogen Idec Inc, Celgene and Exelixis; and specialty and regional pharmaceutical companies and multinational pharmaceutical companies, including but not limited to, Abbott Laboratories Inc., Astellas Pharma Inc., AstraZeneca plc, Bayer-Schering Pharmaceutical, Boehringer Ingelheim AG, Bristol-Myers Squibb, Eisai Co. Ltd., Eli Lilly and Company, F. Hoffmann-LaRoche Ltd., GlaxoSmithKline, Johnson & Johnson, Merck, Novartis, Pfizer, Sanofi-Aventis, Taiho and Takeda Pharmaceutical Co.

## Manufacturing

We do not own or operate manufacturing facilities for the production of MGCD265, mocetinostat or any of our other product candidates, nor do we plan to develop our own manufacturing operations in the foreseeable future. We currently depend on third-party contract manufacturers for all of our required raw materials, API and finished products for our preclinical and clinical trials.

Manufacturers of our products are required to comply with applicable FDA manufacturing requirements contained in the FDA's current good manufacturing practices, or cGMP, regulations. cGMP regulations require, among other things, quality control and quality assurance as well as corresponding maintenance of records and documentation. Pharmaceutical product manufacturers and other entities involved in the manufacture and distribution of approved pharmaceutical products are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMP and other laws. Accordingly, manufacturers must continue to expend time, money, and effort in the area of production and quality control to maintain cGMP compliance. Discovery of problems with a product after approval may result in restrictions on a product, manufacturer or holder of an approved NDA, including withdrawal of the product from the market. In addition, changes to the manufacturing process generally require prior FDA approval before being implemented.

## **Government Regulation**

#### The Regulatory Process for Drug Development

The production and manufacture of our product candidates and our research and development activities are subject to regulation by various governmental authorities around the world. In the United States, drug products are subject to regulation by the FDA. There are other comparable agencies in Canada, Europe and other parts of the world. Regulations govern, among other things, the research, development, testing, manufacture, quality control, approval, labeling, packaging, storage, record-keeping, promotion, advertising, distribution, post-approval monitoring and reporting, marketing and export and import of products. Applicable legislation requires licensing of manufacturing and contract research facilities, carefully controlled research and testing of products, governmental review and/or approval of results prior to marketing therapeutic products. Additionally, adherence to good laboratory practices, or GLP, and good clinical practices, or GCP, during nonclinical and clinical testing and cGMP during production is required. The system of new drug approval in the United States is generally considered to be the most rigorous in the world and is described in further detail below under "U.S. Pharmaceutical Product Development Process." In Canada, these activities are regulated by the Food and Drug Act and the rules and regulations promulgated thereunder, which are enforced by the Therapeutic Products Directorate, or TPD of Health Canada.

## U.S. Pharmaceutical Product Development Process

In the United States, the FDA regulates pharmaceutical products under the Federal Food, Drug and Cosmetic Act and implementing regulations. Pharmaceutical products are also subject to other federal, state and local statutes and regulations. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or after approval, may subject an applicant to administrative or judicial sanctions. FDA sanctions could include refusal to approve pending applications, withdrawal of an approval, a clinical hold, warning letters, product recalls, product seizures, total or partial suspension of production or distribution injunctions, fines, refusals of government contracts, restitution, disgorgement or civil or criminal penalties. Any agency or judicial enforcement action could have a material adverse effect on us.



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It normally takes an average of 10 to 15 years for a typical experimental drug to proceed from concept to approval. The process required by the FDA before a pharmaceutical product may be marketed in the United States generally includes the following:

completion of preclinical laboratory tests and animal studies. The latter often conducted according to GLPs or other applicable regulations, as well as synthesis and drug formulation development leading ultimately to clinical drug supplies manufactured according to cGMPs;

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

performance of adequate and well-controlled human clinical trials according to the FDA's current GCPs, to establish the safety and efficacy of the proposed pharmaceutical product for its intended use;

submission to the FDA of an NDA for a new pharmaceutical product;

potential review by an external advisory committee to the FDA;

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

FDA audit of select preclinical and clinical trial sites that generated the data in support of the NDA; and

FDA review and approval of the NDA.

The lengthy process of seeking required approvals and the continuing need for compliance with applicable statutes and regulations require the expenditure of substantial resources, and approvals are inherently uncertain.

*Preclinical Studies:* Prior to clinical studies, a research phase takes place which involves demonstration of target and function, design, screening and synthesis of inhibitors. Preclinical studies include laboratory evaluations of product chemistry, toxicity and formulation, as well as animal studies to evaluate efficacy and activity, toxic effects, pharmacokinetics and metabolism of the pharmaceutical product candidate and to provide evidence of the safety, bioavailability and activity of the pharmaceutical product candidate in animals. The conduct of the preclinical safety evaluations must comply with federal regulations and requirements including GLPs. The results of the formal IND-enabling preclinical studies, together with manufacturing information, analytical data, any available clinical data or literature as well as the comprehensive descriptions of proposed human clinical studies, are then submitted as part of the IND to the FDA.

The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA places the IND on a clinical hold within that 30-day time period. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. The FDA may also impose clinical holds on a pharmaceutical product candidate at any time before or during clinical trials due to safety concerns or non-compliance. Accordingly, we cannot be certain that submission of an IND will result in the FDA allowing clinical trials to begin, or that, once begun, issues will not arise that suspend or terminate such clinical trial.

*Clinical Trials:* Clinical trials involve the administration of the pharmaceutical product candidate to healthy volunteers or patients under the supervision of qualified investigators, generally physicians not employed by the sponsor. Clinical trials are conducted under protocols detailing, among other things, the objectives of the clinical trial, dosing procedures, subject selection and exclusion criteria, and the parameters to be used to monitor subject safety. Each protocol must be submitted to the FDA if conducted under a U.S. IND. Clinical trials must be conducted in accordance with the FDA's GCP requirements. Further, each clinical trial must be reviewed and approved by an independent institutional review board, or IRB, or ethics committee at or servicing each institution at which the clinical trial will be conducted. An IRB or ethics committee is charged with protecting the welfare and rights of trial participants and considers such items as

whether the risks to individuals participating in the clinical trials are minimized and are reasonable in relation to anticipated benefits. The IRB or ethics committee also approves the informed consent form that must be provided to each clinical trial subject or his or her legal representative and must monitor the clinical trial until completed.

Human clinical trials are typically conducted in three sequential phases that may overlap or be combined:

*Phase 1 Clinical Trials:* Phase 1 clinical trials are usually first-in-man trials, take approximately one to two years to complete and are generally conducted on a small number of healthy human subjects to evaluate the drug's activity, schedule and dose, pharmacokinetics and pharmacodynamics. However, in the case of life-threatening diseases, such as cancer, the initial Phase 1 testing may be done in patients with the disease. These trials typically take longer to complete and may provide insights into drug activity.

*Phase 2 Clinical Trials:* Phase 2 clinical trials can take approximately one to three years to complete and are carried out on a relatively small to moderate number of patients (as compared to Phase 3) in a specific indication. The pharmaceutical product is evaluated to preliminarily assess efficacy, to identify possible adverse effects and safety risks, and to determine optimal dose, regimens, pharmacokinetics, pharmacodynamics and dose response relationships. This phase also provides additional safety data and serves to identify possible common short-term side effects and risks in a larger group of patients. Phase 2 clinical trials sometimes include randomization of patients.

*Phase 3 Clinical Trials:* Phase 3 clinical trials take approximately two to five years to complete and involve tests on a much larger population of patients (several hundred to several thousand patients) suffering from the targeted condition or disease. These studies usually include randomization of patients and blinding of both patients and investigators at geographically dispersed test sites (multi-center trials). These trials are undertaken to further evaluate dosage, clinical efficacy and safety and are intended to establish the overall risk/benefit ratio of the product and provide an adequate basis for product labeling. Generally, two adequate and well-controlled Phase 3 clinical trials are required by the FDA for approval of an NDA or foreign authorities for approval of marketing applications.

*Special Protocol Assessment:* 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. Having an SPA does not guarantee that a product will receive FDA approval.

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

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

in accordance with the IRB's or ethics committee's requirements or if the pharmaceutical product has been associated with unexpected serious harm to patients.

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

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

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

#### **Accelerated Approval**

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

#### **Post-Approval Requirements**

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

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

## FDA Regulation of Companion Diagnostics

As part of our clinical development plans, we are exploring the use of companion diagnostics to identify patients most likely to respond to our product candidates. Companion diagnostics are classified as medical devices under the Federal Food, Drug, and Cosmetic Act in the United States. In the United States, the FDA regulates the medical device design and development, preclinical and clinical testing, premarket clearance or approval, registration and listing, manufacturing, labeling, storage, reporting, recordkeeping, advertising and promotion, export and import, sales and distribution, and post-market surveillance of medical devices. Unless an exemption applies, companion diagnostics require marketing clearance or approval from the FDA prior to commercial distribution. The two primary types of FDA marketing authorization applicable to a medical device are premarket notification, also called 510(k) clearance, and premarket approval, or PMA. According to a 2011 draft guidance issued by FDA officials, companion diagnostics ordinarily will be considered to be high risk and, therefore, will require PMA approval before they are marketed. Some companion diagnostics, however, could potentially be cleared through 510(k) clearance.

The 2011 draft guidance issued by the FDA, if finalized, would address issues critical to developing companion diagnostics, such as biomarker qualification, establishing clinical validity, the use of retrospective data, the appropriate patient population and when the FDA will require that the device and the drug be approved simultaneously. According to the draft guidance, if safe and effective use of a therapeutic product depends on a diagnostic, then the FDA generally will require approval or clearance of the diagnostic at the same time that the FDA approves the therapeutic product. The FDA has yet to issue further guidance, and it is unclear whether it will do so, or what the scope would be.

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

## Other Healthcare Laws and Compliance Requirements

In the United States, our activities are potentially subject to regulation by various federal, state and local authorities in addition to the FDA, including the Centers for Medicare and Medicaid Services and other divisions of the U.S. government, including, the Department of Health and Human Services, the U.S. Department of Justice and individual U.S. Attorney offices within the Department of Justice, and state and local governments. For example, if a drug product is reimbursed by Medicare, Medicaid, or other federal or state healthcare programs, our company, including our sales, marketing and scientific/educational grant programs, must comply with the federal False Claims Act, the federal Anti-Kickback Statute, the federal Physician Payment Sunshine Act and similar state laws and regulations, each as amended from time to time. If a drug product is reimbursed by Medicare or Medicaid, pricing and rebate programs must comply with, as applicable, the Medicaid rebate requirements of the Omnibus Budget Reconciliation Act of 1990, or OBRA, and the Medicare Prescription Drug Improvement and Modernization Act of 2003. Among other things, 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 our approved drug products are made available to authorized users of the Federal Supply Schedule of the General Services Administration, additional laws and requirements may apply.

## Pharmaceutical Coverage, Pricing and Reimbursement

In the United States and markets in other countries, sales of any products for which we receive regulatory approval for commercial sale will depend in part on the availability of coverage and adequate reimbursement from third-party payors, including government authorities, managed care providers, private health insurers and other organizations. In the United States, private health insurers and other third-party payors often provide reimbursement for products and services based on the level at which the government

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(through the Medicare or Medicaid programs) provides reimbursement for such products and services. Third-party payors are increasingly examining the medical necessity and cost-effectiveness of medical products and services in addition to their safety and efficacy and, accordingly, significant uncertainty exists as to the coverage and reimbursement status of newly approved therapeutics. In particular, in the United States, the European Union and other potentially significant markets for our product candidates, government authorities and third-party payors are increasingly attempting to limit or regulate the price of medical products and services, particularly for new and innovative products and therapies, which often has resulted in average selling prices that are lower than they would otherwise be. Further, the increased emphasis on managed healthcare in the United States and on country and regional pricing and reimbursement controls in the European Union will put additional pressure on product pricing, reimbursement and utilization, which may adversely affect our future product sales and regulations related to Medicare, Medicaid and healthcare reform, pharmaceutical reimbursement policies and pricing in general. As a result, coverage and adequate third-party reimbursement may not be available for our product candidates to enable us realize an appropriate return on our investment in research and product development.

The market for our product candidates for which we may receive regulatory approval will depend significantly on access to third-party payors' drug formularies, or lists of medications for which third-party payors provide coverage and reimbursement. The industry competition to be included in such formularies often leads to downward pricing pressures on pharmaceutical companies. Also, third-party payors may refuse to include a particular branded drug in their formularies or may otherwise restrict patient access to a branded drug when a less costly generic equivalent or other alternative is available. In addition, because each third-party payor individually approves coverage and reimbursement levels, obtaining coverage and adequate reimbursement is a time-consuming and costly process. We would be required to provide scientific and clinical support for the use of any product to each third-party payor separately with no assurance that approval would be obtained, and we may need to conduct expensive pharmacoeconomic studies in order to demonstrate the cost-effectiveness of our products. This process could delay the market acceptance of any of our product candidates for which we may receive approval and could have a negative effect on our future revenue and operating results. We cannot be certain that our product candidates will be considered cost-effective. If we are unable to obtain coverage and adequate payment levels for our product candidates from third-party payors, physicians may limit how much or under what circumstances they will prescribe or administer them and patients may decline to purchase them. This in turn could affect our ability to successfully commercialize our products and impact our profitability, results of operations, financial condition, and future success.

Additionally, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively, PPACA, substantially changes the way healthcare is financed by both governmental and private insurers. Among other cost containment measures, PPACA establishes: an annual, nondeductible fee on any entity that manufactures or imports certain branded prescription drugs and biologic agents; a new Medicare Part D coverage gap discount program; and a new formula that increases the rebates a manufacturer must pay under the Medicaid Drug Rebate Program. In the future, there may continue to be additional proposals relating to the reform of the U.S. healthcare system, some of which could further limit coverage and reimbursement of our drug products, once approved.

#### Employees

As of September 30, 2013, we had 38 employees including both permanent and contract employees, and we also utilize the services of consultants on a regular basis. Twenty-seven employees are engaged in product development activities and eleven are in support administration, including business development and finance. None of our employees are represented by labor unions or covered by collective bargaining agreements. On October 1, 2013 we announced that we would be winding down our operations in Montreal, Canada and Princeton, New Jersey. We intend to close operations in New Jersey by the end of October



2013. We expect that our operations in Montreal will transition to San Diego over the next three to six months.

### **Properties**

Our Canadian office is currently located at 7150 Frederick Banting Street, Suite 200, Montreal, Québec, H4S 2A1, and we occupy approximately 10,000 square feet of office and laboratory space. Our U.S. subsidiary is currently located at 125 Village Boulevard, Princeton, New Jersey 08540 with 1,983 square feet of office space. Our corporate headquarters is located at 9363 Towne Centre Drive, San Diego, California 92121 where we occupy approximately 6,800 square feet of office space. The term of our lease at Frederick Banting Street, Montreal expires on August 31, 2014 with an option to extend the lease by six months. The term of our lease at Village Boulevard, Princeton expires on April 30, 2015 with an option to renew the lease for five years. The term of our sublease at Towne Centre Drive, San Diego expires on December 31, 2014. Rental payments are approximately \$13,000 per month for our Montreal office, approximately \$4,000 per month for our Princeton office and approximately \$14,000 per month for our San Diego office. Following the closure of the Princeton and Montreal facilities, our sole location will be in San Diego.

## Legal Proceedings

From time to time we may become involved in legal proceedings or be subject to claims arising in the ordinary course of our business. We are not presently a party to any legal proceedings that in the opinion of our management, if determined adversely to us, would have a material adverse effect on our business, financial condition, operating results or cash flows. Regardless of the outcome, litigation can have an adverse impact on us because of defense and settlement costs, diversion of management resources and other factors.

## **Corporate History**

We were incorporated under the laws of the State of Delaware on April 29, 2013. We are a holding company and primarily conduct our operations through MethylGene Inc., a corporation incorporated under the Canada Business Corporations Act, or MethylGene Canada. On May 8, 2013, we entered into an Arrangement with MethylGene Canada. Subject to the terms and conditions of the Arrangement, the securityholders of MethylGene Canada received one share of our common stock in exchange for every 50 shares of MethylGene Canada pursuant to a court-approved plan of arrangement under the Canada Business Corporations Act. In addition, all outstanding options and warrants to purchase common shares of MethylGene Canada became exercisable on a 50-for-1 basis for shares of our common stock, and a proportionate increase was made to the exercise price or conversion price, as applicable. Upon consummation of the Arrangement on June 28, 2013, MethylGene Canada became our wholly-owned subsidiary. The primary motivations to enter into the Arrangement were to potentially increase trading liquidity and have better access to capital, while reducing the U.S. tax burdens of our significant stockholders. We were also motivated to enhance our U.S. profile with U.S. investors and to be better positioned to attract and retain key personnel.

9222-9129 Québec Inc., formerly MethylGene Inc., or Old MethylGene, was incorporated under Part IA of the Companies Act (Québec) by articles of incorporation dated December 13, 1995. Old MethylGene was party to a court approved arrangement, effective May 19, 2010, under the Companies Act (Québec) involving 1819400 Ontario Inc., 1815303 Ontario Limited, 7503466 Canada Inc. and 7503547 Canada Inc. As part of this arrangement, among other things, 7503466 Canada Inc. and 7503547 Canada Inc. were amalgamated to form MethylGene Canada, which carried on the business of Old MethylGene.



## MANAGEMENT

The following table sets forth information about our executive officers, directors and key employee as of September 30, 2013.

Name	Age	Position
Charles M. Baum, M.D., Ph.D.	55	President and Chief Executive Officer, Director
Mark J. Gergen	51	Executive Vice President and Chief Operations Officer
Isan Chen, M.D.	51	Executive Vice President and Chief Medical and Development Officer
Jamie A. Donadio	38	Vice President, Finance
Rodney W. Lappe, Ph.D. <sup>(3)</sup>	58	Chairman of the Board
Martin Godbout, O.C., Ph.D. <sup>(1)(2)(3)</sup>	57	Director
Henry J. Fuchs, M.D. <sup>(2)(3)</sup>	55	Director
Craig Johnson <sup>(1)</sup>	51	Director
Peter Thompson, M.D. <sup>(1)(2)</sup>	53	Director
Kay Employee		
Key Employee		
James Christensen, Ph.D.	45	Vice President, Research

(1)

Member of the Audit Committee.

## (2)

Member of the Compensation Committee.

#### (3)

Member of the Nominating and Corporate Governance Committee.

## **Executive Officers**

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

Dr. Baum's experience in the pharmaceutical industry provides our Board of Directors with subject matter expertise. In addition, through his position as Chief Medical Officer for Pfizer's Biotherapeutics and Bioinnovation Center, Dr. Baum has acquired the operational expertise which we believe qualifies him to serve on our Board of Directors.

*Mark J. Gergen* has served as our Executive Vice President and Chief Operations Officer since February 2013. From September 2006 to November 2013, he was Senior Vice President, Corporate Development for Amylin Pharmaceuticals, Inc., or Amylin. Starting in January 2005, he was Executive Vice President of CardioNet, Inc. From June 1999 to May 2003, he served as Chief Financial and Development Officer and later Chief Restructuring Officer of Advanced Tissue Sciences, Inc. From August 1994 to June 1999, he was Division Counsel at Medtronic, Inc. Mr. Gergen received a B.A. in Business Administration from Minot State University and a J.D. from the University of Minnesota Law School.

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*Isan Chen, M.D.* has served as our Executive Vice President and Chief Medical and Development Officer since September 2013. Dr. Chen is board certified in Internal medicine, hematology and medical oncology with more than 15 years of experience in oncology and clinical trials from first-in-humans through global registrational studies. He has experience in oncology clinical development and interactions with regulatory agencies in the United States and Europe. He was most recently the Chief Medical Officer of Aragon Pharmaceuticals which was acquired by Johnson & Johnson in July of 2013. At Aragon Pharmaceuticals, Dr. Chen was responsible for the clinical development strategy of all the company's programs, including prostate and breast cancer. Prior to Aragon Pharmaceuticals, Dr. Chen served as Vice President of tumor strategy in the oncology business unit at Pfizer. In addition he was the clinical lead for Sutent, a multiple kinase inhibitor, for the treatment of RCC, an indication in which the drug secured FDA approval in 2006. He was also the clinical lead for the Phase 1 studies of crizotinib and CDK 4/6 inhibitor palbociclib. Dr. Chen completed his hematology/oncology fellowship at University of California, San Diego. Before joining Pfizer, Dr. Chen practiced medicine as a staff physician at City of Hope Medical Center and later as an assistant professor at the University of Texas, M.D. Anderson Cancer Center.

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

#### **Non-Employee Directors**

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

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

*Martin Godbout, O.C., Ph.D.* has served as a member of our Board of Directors since September 2002. Since October 2009, Dr. Godbout has served as the President of Hodran Inc. From April 2000 to October 2009, Dr. Godbout was the Founder, President and Chief Executive Officer of Genome Canada, a private, not-for-profit corporation, dedicated to investing and implementing a national strategy in genomics and proteomics research in Canada. From May 1997 to January 1999, Dr. Godbout was the Senior Vice-President of BioCapital, a Canadian venture capital firm. From May 1994 to May 1997, he was President and General Manager of Société Innovatech Québec, a technology investment fund. In 1994 he founded BioContact Québec, an international biopharmaceutical partnership symposium. From December 1993 to April 1994, he was Assistant Managing Director responsible for biopharmaceutical industry relations at the Research Centre of Centre Hospitalier de l'Université Laval (CHUL). In 1991, Dr. Godbout came back to Laval University as an Assistant Professor at the Department of Psychiatry at the Faculty of Medicine. From 1985 to 1990, he received a postdoctoral fellowship from the Medical Research Council (MRC) of Canada and went to San Diego, California, where he was trained in Neuromolecular Biology at The Scripps Research Institute. Dr. Godbout is presently a member of the Board of Directors of several Canadian

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biopharmaceutical companies, foundations and scientific Canadian organizations, including Acasti Pharma Inc., AmorChem Financial Inc., AngioChem Inc., AsmaCure Ltd., MethylGene Inc. (chairman), Génome Québec (chairman), BioContact, BioQuébec FQRS, Montréal In Vivo et la Fondation de l'ataxie de Charlevoix. Dr. Godbout has been a member of the Board of Directors of the "Conseil de la Science et de la Technologie du Québec" from 1996 to 2004 and of the National Science and Engineering Research Council of Canada from 1999 to 2002. Dr. Godbout holds a B.Sc. in biochemistry (1979) and a Ph.D. in physiology and molecular endocrinology from Laval University in Québec City.

Based on Dr. Godbout's experience in the biopharmaceutical industry and his scientific background, we believe Dr. Godbout has the appropriate set of skills to serve on our Board of Directors.

*Craig Johnson* has served on our Board of Directors since September 2013 and as Chairman of the Audit Committee since that time. Mr. Johnson is currently a director and chairman of the audit committee for Adamis Pharmaceuticals Corporation and a director at La Jolla Pharmaceutical Company. Mr. Johnson also served as a director and chairman of the audit committee for Ardea Biosciences, Inc., from 2008 until its sale to AstraZeneca PLC in 2012. He was Chief Financial Officer of PURE Bioscience, Inc. from 2011 to 2012, and Senior Vice President and Chief Financial Officer of NovaDel Pharma Inc. from 2010 to 2011. Mr. Johnson served as Vice President and Chief Financial Officer of TorreyPines Therapeutics, Inc. from 2004 until its sale to Raptor Pharmaceuticals Corp. in October 2009, and then as Vice President of a wholly-owned subsidiary of Raptor Pharmaceutical Corp. through March 2010. He also held several positions, including Chief Financial Officer and Senior Vice President of Operations, at MitoKor, Inc. from 1994 to 2004. Prior to 1994, Mr. Johnson held similar positions with several early-stage technology companies, and he also practiced as a Certified Public Accountant with Price Waterhouse. Mr. Johnson received his B.B.A. in accounting from the University of Michigan.

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

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

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

*Peter Thompson, M.D.* has served as a member of our Board of Directors since June 2011. Since September 2013 he has been a Private Equity Partner at Orbimed Advisors LLC, a healthcare dedicated investment firm, where he previously served as a Venture Partner from August 2010 through September 2013. In 2002, he co-founded Trubion Pharmaceuticals, and served as its Chief Executive Officer and Chairman until 2009. Dr. Thompson is the former Vice President & General Manager of Chiron Informatics at Chiron Corporation and held various executive positions in Becton, Dickinson, and Company, including Vice President, Research and Technology Department. He serves as a director on the Boards of Anthera Pharmaceuticals Inc., Response Biomedical Inc., Cleave Biosciences Inc. (co-founder) and Principia Biosciences Inc. Dr. Thompson is an Ernst & Young LLP Entrepreneur of the Year awardee, an inventor of numerous patents, a board-certified internist and oncologist, and an Affiliate Professor of Neurosurgery at the University of Washington. He was on faculty at the National Cancer Institute, trained in internal medicine training at Yale University, and received his M.D. from Brown University.

We believe Dr. Thompson's leadership and experience in the pharmaceutical industry and his success as a venture capitalist qualify him to serve on our Board of Directors.

#### **Key Employee**

*James Christensen, Ph.D.* has served as our Vice President, Research since June 2013. He was most recently the Senior Director of Oncology Precision Medicine in the Oncology Research Unit at Pfizer, a group focused on developing strategies toward patient identification, novel combination approaches, and development of biomarker approaches. Dr. Christensen joined Pfizer in 2003 and his responsibilities there included leading nonclinical research efforts for oncology programs including sunitinib malate research activities and leading the nonclinical and translational biology efforts for other research and development programs including crizotinib. Dr. Christensen participated as a member of the Pfizer Cancer Research or Oncology Research Unit leadership team from 2005 to 2013. Prior to 2003, Dr Christensen was at SUGEN/Pharmacia as a Group Leader on the Preclinical Research and Exploratory Development team where he was responsible for leadership of Met and erbB family preclinical development programs and aspects of research and development for Sutent. Dr. Christensen initiated his industry experience at Warner Lambert/Parke-Davis with research focus in RTK biology and RTK pathway biomarker development in the oncology therapeutic area. Dr. Christensen has authored or co-authored over 100 peer-reviewed research articles in scientific journals including Science, Nature, Cancer Cell, New England Journal of Medicine and others. In addition, Dr. Christensen participates on the editorial boards for Cancer Research and Molecular Cancer Therapeutics. Dr. Christensen received his Ph.D. degree focusing in Molecular Pharmacology from North Carolina State University with dissertation research directed toward characterization of mechanisms of apoptosis dysregulation during the process of carcinogenesis.

#### **Board Composition**

Our business and affairs are organized under the direction of our Board of Directors, which currently consists of six members. The primary responsibilities of our Board of Directors are to provide oversight, strategic guidance, counseling and direction to our management. Our Board of Directors meets on a regular basis and additionally as required.

Our Board of Directors has determined that five of our six directors, Martin Godbout, O.C., Ph.D., Henry J. Fuchs, M.D., Rodney W. Lappe, Ph.D., Craig Johnson and Peter Thompson, M.D., are independent directors, as defined by Rule 5605(a)(2) of the NASDAQ Listing Rules.

Each director serves until the next annual meeting of stockholders following such director's election to the Board or Directors and until their successors are duly elected and qualified. The authorized size of our Board of Directors is currently six members. The authorized number of directors may be changed only by resolution of the Board of Directors. Our directors may be removed for cause by the affirmative vote of the holders of at least a majority of our voting stock.

## **Board of Directors Leadership Structure**

The Board of Directors has a Chairman of the Board, Dr. Lappe, who has authority, among other things, to call and preside over Board of Directors meetings, to set meeting agendas, and to determine materials to be distributed to the Board of Directors. Accordingly, the Chairman has substantial ability to shape the work of the Board of Directors. In addition, we have a separate chair for each committee of the Board of Directors. The chairs of each committee are expected to report annually to the Board of Directors on the activities of their committee in fulfilling their responsibilities as detailed in their respective charters or specify any shortcomings should that be the case. We believe that separation of the positions of Chairman and Chief Executive Officer reinforces the independence of the Board of Directors in its oversight of our business and affairs. In addition, we believe that having a separate Chairman creates an environment that is more conducive to objective evaluation and oversight of management's performance, increasing management accountability and improving the ability of the Board of Directors to monitor whether management's actions are in our best interests and the best interests of our stockholders. As a result, we believe that having a separate Chairman can enhance the effectiveness of the Board of Directors as a whole.



### Role of the Board of Directors in Risk Oversight

The Audit Committee of the Board of Directors is primarily responsible for overseeing our risk management processes on behalf of the Board of Directors. Going forward, we expect that the Audit Committee will receive reports from management at least quarterly regarding our assessment of risks. In addition, the Audit Committee reports regularly to the Board of Directors, which also considers our risk profile. The Audit Committee and the Board of Directors focus on the most significant risks we face and our general risk management strategies. While the Board of Directors oversees our risk management, management is responsible for day-to-day risk management processes. Our Board of Directors expects management to consider risk and risk management in each business decision, to proactively develop and monitor risk management strategies and processes for day-to-day activities and to effectively implement risk management strategies adopted by the Audit Committee and the Board of Directors. We believe this division of responsibilities is the most effective approach for addressing the risks we face and that our Board of Directors leadership structure, which also emphasizes the independence of the Board of Directors in its oversight of its business and affairs, supports this approach.

#### **Board of Directors Committees**

Our Board of Directors has established three committees, each with its own charter and each committee is comprised of independent directors.

#### Audit Committee

The principal duties of the Audit Committee of the Board of Directors include assisting the Board of Directors in its oversight of:

the quality and integrity of our financial statements and reports;

our accounting and financial reporting process, system of internal controls over financial reporting and audit process;

compliance with, and process for monitoring compliance with, legal and regulatory requirements;

the independent auditors' qualifications, independence and performance;

our legal, regulatory and ethical compliance programs as established by management and the Board of Directors; and

pre-approval of all audit and non-audit services provided by the independent registered public accounting firm.

The current members of the Audit Committee are Mr. Johnson (chair), Dr. Godbout and Dr. Thompson. Our Board of Directors has determined that each member of the Audit Committee is an independent director under NASDAQ Listing Rules and under Rule 10A-3 under the Exchange Act. Each member of our Audit Committee can read and understand fundamental financial statements in accordance with NASDAQ audit committee requirements and is financially literate, as required by Canadian securities laws. In arriving at this determination, the Board of Directors has examined each Audit Committee member's scope of experience and the nature of their employment in the corporate finance sector.

Our Board of Directors has determined that Mr. Johnson qualifies as an audit committee financial expert within the meaning of SEC regulations and meets the financial sophistication requirements of the NASDAQ Listing Rules. In making this determination, our Board of Directors has considered formal education and the nature and scope of experience each has previously had with public companies. Both our independent registered public accounting firm and management periodically meet privately with our Audit Committee.

The Audit Committee charter can be found on our website at www.mirati.com in the Corporate Governance section. The inclusion of our website address in this Registration Statement does not include or incorporate by reference the information on our website.

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#### **Compensation Committee**

The principal duties of the Compensation Committee of the Board of Directors include:

reviewing and approving our overall compensation strategy and policies;

reviewing and approving corporate performance goals, compensation and other terms of employment of our executive officers;

reviewing the compensation of our non-employee directors;

administering our stock option and purchase plans; and

preparing the annual report on executive compensation for purposes of disclosure to our stockholders.

In addition, the Compensation Committee reviews and approves overall compensation strategies and policies. In exercising these duties the Compensation Committee ensures that our compensation programs, particularly in connection with bonus targets, is aligned with the interests of our stockholders and other stakeholders. The majority of the named executive officers' bonus targets are based on corporate-based goals that strive to increase stockholder value and, to a lesser extent, to individual goals that help drive the corporate goals. The Compensation Committee regularly enlists the services of a third-party company to conduct an evaluation of current market practices to benchmark against our current practices. The last such review was undertaken by Towers Watson in May 2011.

The current members of the Compensation Committee are Dr. Godbout (chair), Dr. Fuchs and Dr. Thompson. Our Board of Directors has determined that all such members are independent under the NASDAQ Listing Rules and Canadian securities laws, are "non-employee directors" as defined in Rule 16(b)-3 promulgated under the Exchange Act and are "outside directors" as that term is defined in Section 162(m) of the Code.

The Compensation Committee charter can be found on our website at www.mirati.com in the Corporate Governance section. The inclusion of our website address in this Registration Statement does not include or incorporate by reference the information on our website.

#### Nominating and Corporate Governance Committee

The principal duties of the Nominating and Corporate Governance Committee of the Board of Directors are to develop and implement a set of corporate governance principles and policies, including a code of business conduct and ethics, assess the performance of the Board of Directors, its committees and the contributions of individual directors, and review and oversee management succession planning. As part of this process the Nominating and Corporate Governance Committee periodically reviews and assesses these policies and principles and their application and recommends to the Board of Directors any changes to such policies and principles. The principal duties of the Nominating and Corporate Governance Committee in connection with the nomination of directors are to evaluate the size of the Board of Directors; identify the skill sets currently available and skill sets that may be required; and recommend to the Board of Directors the director nominees to be put before the stockholders at our annual meeting.

The current members of the Nominating and Corporate Governance Committee are Dr. Godbout (chair), Dr. Fuchs and Dr. Lappe. Our Board of Directors has determined that all such members are independent under the NASDAQ Listing Rules and Canadian securities laws, are "non-employee directors" as defined in Rule 16(b)-3 promulgated under the Exchange act and are "outside directors" as that term is defined in Section 162(m) of the Code.

The Nominating and Corporate Governance Committee charter can be found on our website at www.mirati.com in the Corporate Governance section. The inclusion of our website address in this Registration Statement does not include or incorporate by reference the information on our website.

## EXECUTIVE COMPENSATION

#### Overview

The Compensation Committee of the Board of Directors administers our compensation programs on behalf of the Board of Directors. Although focused on executive compensation, the Compensation Committee also sets the annual compensation guidelines for all employees. The Compensation Committee has a charter that is reviewed and updated annually, or as may be warranted from time to time. The members of the Compensation Committee are Dr. Martin Godbout (Chair), Dr. Henry J. Fuchs and Dr. Peter Thompson.

This section addresses the compensation of:

Dr. Charles M. Baum, President and Chief Executive Officer;

Dr. Rachel W. Humphrey, former Executive Vice President and Chief Medical Officer, whose employment ended on September 30, 2013;

Dr. Jeffrey M. Besterman, former Executive Vice President of Research & Development and Chief Scientific Officer, who resigned in April 2013; and

Charles Grubsztajn, former President and Chief Executive Officer, who resigned in September 2012.

The above executive officers are collectively referred to as the named executive officers.

The elements of the compensation program for the named executive officers include: base salary; a non-equity incentive plan; a long-term, equity-based incentive plan; and other compensation, including certain health, welfare and retirement benefits and when determined necessary, limited perquisites. The named executive officers also have termination and change of control benefits in their respective employment agreements (see "Potential Payments Upon Termination or Change of Control" and "Employment Agreements" below).

## **Base Salary**

The compensation of our named executive officers is generally determined and approved by our Board of Directors, based on the recommendation of the Compensation Committee. Our Board of Directors approved the following 2012 base salaries for our named executive officers:

Name	Base Salary
Dr. Baum	\$ 500,000
Dr. Humphrey	\$ 350,000
Dr. Besterman	\$ 308,284
Mr. Grubsztajn	\$ 310,000

Prior to his appointment as President and Chief Executive Officer in November 2012, Dr. Baum was paid consulting fees totaling \$67,885 in 2012.

#### Non-Equity Incentive Plan Bonus

Our named executive officers are eligible to receive annual performance-based cash bonuses. The annual performance-based bonus each named executive officer is eligible to receive is based on (1) the individual's target bonus, as a percentage of base salary, (2) our achievement of corporate goals and (3) the named executive officers' achievement of individual goals.

The maximum bonus that each named executive officer can earn is typically based on the named executive officer's title and guideline ranges set by the Board of Directors. The maximum bonus that each of the named executive officer could earn for 2012 as a percentage of their base salary,

or maximum bonus percentage, were as follows: 40% for Dr. Humphrey; 35% for Dr. Besterman (who is entitled to a minimum bonus of 10% of his base salary); and 50% for Mr. Grubsztajn. With the exception of Dr. Besterman, there is no minimum bonus established for the named executive officers. Dr. Baum was not eligible to receive a

bonus in 2012 because he commenced his employment in November 2012. Mr. Grubsztajn was not eligible to receive a bonus in 2012 because he resigned in September 2012. However, under the terms of his Termination Agreement and Release, Mr. Grubsztajn received a cash payment of \$136,705 in respect of his 2012 bonus.

In early 2012, the Compensation Committee established both corporate and individual bonus goals for the named executive officer bonus awards, which were more heavily dependent upon the achievement of corporate goals than individual goals. The corporate goals were to implement and manage each of our two lead programs (MGCD265 and MGCD290), meet timelines and quality standards and prepare the clinical development plan and budget for subsequent studies for such programs and to achieve an increase in our share price. The individual bonus goals varied for each individual named executive officer and included identifying, selecting and developing biomarkers for MGCD265 and conducting preclinical studies that could impact future clinical development; and presenting and implementing a new clinical organizational plan and initiating further trials with MGCD290. In early 2013, the Compensation Committee considered our overall performance and the performance of each named executive officer and determined that several of the corporate and individual goals had not been met but other goals had been met or exceeded through important events, such as the completion of the private placement in November 2012, and good progress on MGCD290, including our achievement of top line results for the randomized Phase 2 trial in March 2013. Therefore, the Compensation Committee made a recommendation to the Board of Directors based on a subjective review of all the corporate and individual goal achievements in determining the final bonus payouts to the named executive officers for 2012. The Board of Directors approved the following bonus payments:

Dr. Humphrey was awarded a bonus of \$84,000 largely because of the progress on MGCD290, on which her corporate and individual goals were primarily dependent; and

Dr. Besterman was awarded a bonus of \$30,828 because although progress was made on MGCD290, Dr. Besterman did not fully achieve his individual goals.

#### Long-term Incentive Program

In connection with the long-term stock option award program, we use stock options to incentivize the named executive officers over a number of years. The exercise price, vesting and term of the stock options awarded are based on the terms of the Stock Option Plan. The Compensation Committee often makes initial stock option grants upon an executive's commencement of employment and may make annual stock option grants to some or all executives. The initial level of the long-term equity component is determined on a case-by-case basis and is more subjective than the other components of compensation. In determining the initial option award, the Board of Directors considers the most recent market evaluations that it has commissioned and other factors such as the candidate's expectations and any unique situation that may exist at the time of hiring. Annual stock option awards are determined by the Board of Directors based on availability of options, performance, current individual holdings and overall compensation.

In 2012, the Compensation Committee approved the following stock option award grants to the named executive officers. In connection with his commencement of employment as President and Chief Executive Officer, and pursuant to his employment agreement, Dr. Baum was granted an option to purchase 190,760 shares on November 13, 2012 at an exercise price of CND\$8.50 (or US\$8.51, as converted as of the date of grant of such option) per share. Pursuant to the terms of his employment agreement as further described below, Dr. Baum was awarded additional stock options to purchase 207,240 shares on July 17, 2013 upon the effectiveness of our 2013 Plan. In connection with her commencement of employment, Dr. Humphrey was granted an option to purchase 41,328 shares on January 4, 2012 at an exercise price of CND\$15.50 (or US\$15.30, as converted as of the date of grant of such option) per share and an option to purchase 41,335 shares on July 17, 2012 at an exercise price of CND\$12.50 (or US\$12.32, as converted as of the date of grant of such option) per share. On July 17, 2012, Dr. Besterman was granted an option to purchase 26,811 shares and Mr. Grubsztajn was granted an option to purchase 78,128 shares, in both cases at an exercise price of CND\$12.50 (or US\$12.32, as converted as of the date of grant of such option) per share. All of the stock options granted in 2012 vest 20% on the date of grant and 20% on each



of the next four anniversary dates, subject to the named executive officer's continued service with us through such dates.

## Perquisites, Health, Welfare and Retirement Benefits

Our named executive officers are eligible to participate in all of our employee benefit plans, including our medical, dental, group life and disability insurance plans and our retirement plans, which are funded entirely by us, with the exception of the life insurance premiums, which are funded equally by our employees and us, and are provided to the named executive officers on the same basis as other employees. The retirement plans we sponsor are a registered retirement savings program, or RRSP, for Canadian-based employees and, beginning effective January 1, 2013, a 401(k) plan for U.S.-based employees.

The RRSP is a personal retirement plan for Canadians that provides for tax deductions under Canadian tax law. We offer our Canadian-based employees a benefit to match 5% of an employee's salary, up to a maximum of CND\$2,500 per year, in the employee's personal RRSP account. Individuals may choose to contribute up to a maximum contribution of 18% of annual salary subject to a maximum annual contribution of CND\$22,500 (for 2012) into their personal RRSP account. The 401(k) plan is a retirement savings defined contribution plan established in accordance with Section 401 of the Code that provides our U.S.-based employees with the opportunity to defer their eligible compensation on a pre-tax basis, up to statutorily prescribed annual limits and have this amount contributed to the 401(k) plan. In 2013, we will provide a matching contribution of 4% up to a maximum contribution of CND\$2,500.

The named executive officers generally do not receive any material perquisites. However, Dr. Baum, Dr. Humphrey and Dr. Besterman receive payments to equalize their taxes between Canadian and U.S. tax rates. Each of Dr. Baum, Dr. Humphrey and Dr. Besterman receive an annual grossed-up payment from us representing the difference between (1) the aggregate income taxes due and payable in Canada (federal plus provincial) and in the United States (federal plus state) and (2) the aggregate income taxes they would have otherwise been due and payable in the United States (federal plus state) had the executive not been required to pay income taxes in Canada. Dr. Besterman was also entitled to have his tax preparation costs for his annual tax returns paid by us and a flexible benefit payment equal to 10% of his base salary to be used for travel, insurance coverage, educational needs or retirement programs under the terms of his employment agreement.

## Summary Compensation Table

The following table presents summary information regarding the total compensation awarded to, earned by, or paid to each of our named executive officers for services rendered in all capacities for the year ended December 31, 2012.

Name & Principal Position Charles M. Baum, M.D., Ph.D.,	<b>Year</b> 2012	<b>Salary</b> \$135,438	Bonus \$	Option- based	-	All Other	Total Compensation \$ 1,445,262
President and Chief Executive Officer(2)	2012	¢ 155, 150	Ψ	¢1,509,021	Ψ	Ψ	φ 1,113,202
Rachel Humphrey, M.D., Former Executive Vice President and Chief Medical Officer(3)	2012	350,000	275,000	888,324	84,000	26,637	1,623,961
Jeffrey M. Besterman, Ph.D., Former Executive Vice President, Research & Development and Chief Scientific Officer(4)	2012	308,284		253,589	30,828	126,013	718,714
Charles Grubsztajn, Former President and Chief Executive Officer(5)	2012	255,808		739,192		466,054	1,461,054

(1)

In accordance with SEC rules, this column reflects the aggregate grant date fair value of the option awards granted during 2012 computed in accordance with Financial Accounting Standard Board Accounting Standards Codification Topic 718 for stock-based compensation transactions, or ASC 718. Assumptions used in the calculation of these amounts are included in Note 13 to our consolidated financial statements appearing elsewhere in this prospectus. These amounts do not reflect the actual economic value that will be realized by the named executive officer upon the vesting of the stock options, the exercise of the stock options, or the sale of the shares of common stock underlying such stock options. The value of all option awards in the table above using the average monthly U.S. dollar per Canadian dollar conversion rate from the Bank of Canada for the month in which the grant date occurred, which was 0.9869, 0.9863 and 1.0030 for January, July and November 2012 grant dates, respectively.

(2)

Dr. Baum commenced employment with us in November 2012 as our President and Chief Executive Officer. Dr. Baum served as a consultant from September 24, 2012 through November 8, 2012. Dr. Baum's base salary is paid in U.S. dollars and the amount listed in the "Salary" column for Dr. Baum includes \$67,885 paid to him during 2012 in consulting fees. Dr. Baum's consulting fees were paid in Canadian dollars and converted to the U.S. dollar amount in the table above using the weekly average U.S. dollar per Canadian dollar conversion rate from the Bank of Canada during the period of his consulting contract, which was 1.0086.

# (3)

Dr. Humphrey joined us in January 2012 and her employment was terminated effective as of September 30, 2013. Dr. Humphrey's base salary and bonus were paid in U.S. dollars. The "Bonus" column for Dr. Humphrey reflects a signing bonus of \$275,000 paid to Dr. Humphrey pursuant to her employment agreement. The "All Other Compensation" column for Dr. Humphrey reflects a tax equalization payment of \$10,623 that relates to her 2012 compensation, including a related gross up payment of \$16,014, as further described under "Employment Agreements" below. Dr. Humphrey's tax equalization and gross up payment was paid in Canadian dollars and converted to the U.S. dollar amount in the table above using the average weekly U.S. dollar per Canadian dollar conversion rate from the Bank of Canada for 2012, which was 1.0006.

# (4)

Dr. Besterman resigned in April 2013. Dr. Besterman's compensation was paid in U.S. dollars except as noted below. The "All Other Compensation" column for Dr. Besterman reflects (1) matching contributions to the RRSP in the amount of \$2,502, (2) the flexible benefit payment pursuant to his employment agreement in the amount of \$30,828, and (3) a tax equalization payment that relates to his 2012 compensation, and a related gross up payment of \$41,847. The tax equalization payment and flexible benefit payments were pursuant to the terms of Dr. Besterman's employment agreement as further described under "Employment Agreements" below. Dr. Besterman's RRSP contribution tax equalization and gross up payment was paid in Canadian dollars and converted to the U.S. dollar amount in the table above using the average weekly U.S. dollar per Canadian dollar conversion rate from the Bank of Canada for 2012, which was 1.0006.

# (5)

Mr. Grubsztajn resigned in September 2012. The amount listed in "Salary" column for Mr. Grubsztajn represents the salary and vacation earned by and paid to Mr. Grubsztajn, through his resignation date. The "All Other Compensation" column

represents the value of severance benefits (base salary, bonus, legal fees and continued benefits) provided to Mr. Grubsztajn in accordance with the terms of his termination agreement and release, as further described under " Employment Agreements" below. Mr. Grubsztajn's compensation was paid in Canadian dollars and converted to the U.S. dollar amounts in the table above using, for base salary and bonus payments, the average weekly U.S. dollar per Canadian dollar conversion rate from the Bank of Canada over the period payments were made, which was 0.9980 and for termination payments, the average monthly U.S. dollar per Canadian dollar conversion rate from the severance payment was made, which was 1.0222.

## Outstanding Equity Awards at Fiscal Year-End

The following table presents for each named executive officer, information regarding outstanding stock options held as of December 31, 2012.

		Option Awards Number of Number of securities securities underlying underlying				
		options	unexercised options	Option exercise	Option expiration	
Name	Grant Date	-	nexercisable(3		date	
Charles M. Baum, M.D.	11/13/2012	38,152	152,608	\$ 8.51	11/12/2017	
Rachel Humphrey, M.D.	01/04/2012	8,266	33,062	15.30	01/3/2017	
	07/17/2012	8,267	33,068	12.32	07/16/2017	
Jeffrey M. Besterman,						
Ph.D.	02/20/2004	204		152.87	02/20/2014	
	06/29/2004	2,450		157.74	06/28/2014	
	12/15/2004	700		125.44	12/14/2014	
	03/11/2008	350		113.05	03/10/2013	
	12/18/2008	1,500		7.08	12/17/2013	
	03/31/2009	700		14.42	03/30/2014	
	07/21/2011	10,667	10,667	17.57	07/20/2016	
	07/17/2012	5,363	21,449	12.32	07/16/2017	
Charles Grubsztajn <sup>(2)</sup>						

(1)

The options have not been, and may never be, exercised and actual gains, if any, on exercise will depend on the value of the shares of common stock on the date of exercise.

(2)

All options that were previously awarded to Mr. Grubsztajn expired in connection with his termination and he had no outstanding options as December 31, 2012.

(3)

The 10,667 shares underlying unexercisable options held by Dr. Besterman were granted on July 21, 2011 and have a vesting schedule of 25% on the date of grant and 25% on each of the next three anniversary dates so that the option will be fully vested on the four year anniversary of the grant date, subject to Dr. Besterman's continued service through each such vesting date. All other unexercisable options reflected in the above table were granted in 2012 and vest 20% on the date of grant and 20% on each of the next four anniversary dates so

# Explanation of Responses:

that the option will be fully vested on the four year anniversary of the grant date, subject to the executive's continued service through each such vesting date.

(4)

The exercise price reflected above is converted from the closing sale price of our shares of common stock on the TSX on the day before the date of grant to U.S. dollars using the U.S. dollar per Canadian dollar conversation rate from the Bank of Canada for the date of grant.

We did not engage in any repricings or other modifications or cancellations to any of our named executive officers' outstanding option awards during the year ended December 31, 2012.

## **Employment Agreements**

We have entered into employment agreements with each of our named executive officers, as further described below. The employment agreements provide that: (1) the officer will receive a base salary; (2) the officer will be eligible to receive an annual performance-based bonus; and (3) the officer will be eligible to receive grants of stock options which will be reviewed annually in accordance with our policies and will be eligible to participate in our fringe benefit programs. The employment agreements have an indefinite term.

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Furthermore, the employment agreements provide for, among other things, specific non-competition and non-solicitation covenants, which remain in effect for one year following termination, as well as a confidentiality covenant which remains in effect indefinitely or until the confidential information is publicly disclosed. In addition, there are covenants stipulating that any intellectual property developed in the course of their employment is our property.

#### Dr. Baum

*Employment Agreement Prior to Arrangement.* We entered into an employment agreement with Dr. Baum in November 2012 in connection with his appointment as President and Chief Executive Officer. Prior to this appointment, Dr. Baum served as a consultant to us from September 2012 to November 2012. The employment agreement provided for:

an annual base salary of \$500,000;

an annual non-equity incentive plan bonus of up to 50% of his annual base salary;

an initial equity component of 398,000 options representing 4% of the total outstanding shares immediately after the private placement financing which closed on November 21, 2012 of which Dr. Baum was awarded options to purchase approximately 190,760 shares on November 13, 2012 and received the remainder of the equity component of his compensation representing options to purchase 207,240 shares on July 17, 2013 upon the effectiveness of the 2013 Plan; and

participation in our fringe benefit programs that are available to all U.S.-based employees, which include health benefits and a 401(k) plan.

Dr. Baum was also entitled to annual tax equalization payments equal to the difference between the aggregate Canadian and U.S. taxes due and payable by Dr. Baum as a result of his compensation and the aggregate U.S. taxes that would otherwise have been due if Dr. Baum had not been required to pay Canadian taxes. We pay this amount after the end of each calendar year and will also pay a gross up to Dr. Baum on the equalization payment.

*Employment Agreement After the Arrangement.* In connection with the consummation of the Arrangement, we entered into an amended and restated employment agreement with Dr. Baum in July 2013 that replaced and superseded Dr. Baum's prior employment agreement described above. The amended and restated employment agreement governs Dr. Baum's services to us following the Arrangement and makes certain clarifications and updates for applicable law. Under the amended and restated employment agreement, Dr. Baum serves as our President and Chief Executive Officer and is entitled to an annual base salary of \$500,000 and an annual non-equity incentive plan bonus target of 50% of his annual base salary. Dr. Baum is entitled to generally the same benefit programs and annual tax equalization payments described above under his prior employment agreement and the remainder of the equity component of his compensation representing options to purchase 207,240 shares. The amended and restated employment agreement clarifies that Dr. Baum's employment is at will and may be terminated at any time by either us or Dr. Baum.

Dr. Baum also is entitled to termination benefits that are described in the "Potential Payments Upon Termination or Change of Control" below.

#### Dr. Humphrey

*Employment Agreement Prior to the Arrangement.* We entered into an employment agreement with Dr. Humphrey in January 2012 which provided for:

an annual base salary of \$350,000;

an annual non-equity incentive plan bonus up to 40% of her annual base salary;

a signing bonus of \$275,000; and

participation in our fringe benefit programs that are available to all U.S.-based employees, which include health benefits and a 401(k) plan.

For 2012, we also provided Dr. Humphrey with annual tax equalization payments similar to those provided to Dr. Besterman.

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*Employment Agreement After the Arrangement.* In connection with the consummation of the Arrangement, we entered into an amended and restated employment agreement with Dr. Humphrey in July 2013 that replaced and superseded Dr. Humphrey's prior employment agreement described above. The amended and restated employment agreement governed Dr. Humphrey's services to us following the Arrangement and made certain clarifications and updates for applicable law. Under the amended and restated employment agreement, Dr. Humphrey served as our Executive Vice President and Chief Medical Officer and was entitled to an annual base salary of \$350,000 and an annual non-equity incentive plan bonus target of 40% of her annual base salary. Dr. Humphrey was entitled to generally the same benefit programs described above under her prior employment agreement, except for the signing bonus, which we previously paid to Dr. Humphrey. The amended and restated employment agreement clarified that Dr. Humphrey's employment was at will and could be terminated at any time by either us or Dr. Humphrey.

*Separation Agreement.* In connection with her termination of employment in September 2013, we entered into a separation agreement and release with Dr. Humphrey that superseded the terms of her employment agreement. Under the separation agreement, in exchange for a release of claims against us, Dr. Humphrey received total compensation of approximately \$362,007, which represented:

an amount of \$350,000, which was equal to 12 months of her base salary, payable in substantially equal installments on our regular payroll schedule during the 12 month period following her termination; and

an amount of \$12,007 to cover her ongoing medical coverage under our health insurance plans for 12 months after her termination.

Pursuant to the terms of our Stock Option Plan, all of Dr. Humphrey's non-vested options expired on September 30, 2013 and all of Dr. Humphrey's unexercised options that had vested on the date of her termination will terminate on December 29, 2013.

#### Dr. Besterman

*Employment Agreement.* We entered into an employment agreement with Dr. Besterman in January 1999 that was amended most recently in December 2008. The employment agreement provided for:

an annual base salary of \$270,000, which may be increased each year;

a flexible benefit payment of 10% of base salary to be used for travel, insurance coverage, educational needs or retirement programs, an annual non-equity incentive plan bonus up to 35% and not less than 10% of his annual base salary;

participation in our fringe benefit programs that are available to all employees, which include health benefits and the RRSP;

assistance and payment to prepare income tax returns and Canadian employment forms; and

reimbursement for French language lessons.

Dr. Besterman was also entitled to annual tax equalization payments and related tax gross up payments similar to the benefits described above for Dr. Baum and Dr. Humphrey. Dr. Besterman was also entitled to certain termination benefits.

*Termination Agreement.* In connection with Dr. Besterman's termination in April 2013, we entered into a new agreement with him that superseded the terms of his employment agreement and provided the following termination benefits: a \$679,072 payment equivalent to two years of base salary plus fringe benefits; a \$50,000 lump sum payment for moving and travel expenses; a \$115,000 lump sum payment to cover tax equalization, and tax gross up and tax liability resulting from Dr. Besterman's deemed distribution of certain assets and continued health benefit coverage for one year following his termination date.

Pursuant to the terms of our Stock Option Plan, all of Dr. Besterman's non-vested options expired on April 13, 2013 and all of Dr. Besterman's unexercised options that had vested on the date of his termination terminated on July 12, 2013.

#### Mr. Grubsztajn

*Employment Agreement.* We entered into an employment agreement with Mr. Grubsztajn in May 2005, that was amended most recently in May 2011, which provided for:

a base salary of \$260,000 Canadian dollars, which may be increased each year;

a non-equity incentive plan bonus of up to 50% of his annual base salary; and

participation in our Stock Option Plan.

*Termination Agreement.* We entered into a termination agreement and release with Mr. Grubsztajn upon his resignation in September 2012 that superseded the terms of his employment agreement. Under the termination agreement and release, in exchange for a release of claims against us, Mr. Grubsztajn received total compensation of \$466,054 (calculated using an exchange rate of 1.0222, the exchange rate as of the date such payments were made), which represented:

a \$316,878 lump sum payment, which was equal to 12 months of his base salary;

a \$136,705 lump sum payment that the Board of Directors determined should be paid in respect of his 2012 bonus;

a \$7,360 payment for his legal fees; and

an amount of \$5,111 to cover his ongoing medical coverage under our medical, dental and life insurance plans, excluding short term and long term disability, for 12 months after the resignation date.

Pursuant to the terms of our Stock Option Plan, all of Mr. Grubsztajn's non-vested options expired on September 21, 2012 and all of Mr. Grubsztajn's unexercised options that had vested on the date of his termination terminated on December 20, 2012.

#### Mr. Gergen

*Employment Agreement Prior to the Arrangement.* We entered into an employment agreement with Mr. Gergen in February 2013, which provided for:

an annual base salary of \$375,000;

a non-equity incentive plan bonus up to 40% of his annual base salary;

an initial stock option award to purchase 132,000 shares which he received on July 17, 2013 upon the effectiveness of the 2013 Plan; and

participation in our fringe benefit programs that are available to all U.S.-based employees, which include health benefits and a 401(k) plan.

*Employment Agreement After the Arrangement.* In connection with the consummation of the Arrangement, we entered into an amended and restated employment agreement with Mr. Gergen in July 2013 that replaces and supersedes his prior employment agreement described above. The amended and restated employment agreement governs Mr. Gergen's services to us following the Arrangement and makes certain clarifications and updates for applicable law. Under the amended and restated employment agreement, Mr. Gergen serves as our Executive Vice President and Chief Operations Officer and is entitled to an annual base salary of \$375,000 and an annual non-equity incentive plan bonus target of 40% of his annual base salary. Mr. Gergen is entitled to generally the same benefit programs described above under his prior employment agreement. The amended and restated employment agreement clarifies that Mr. Gergen's employment is at will and may be terminated at any time by either us or Mr. Gergen.

Mr. Gergen also is entitled to receive termination benefits that are described in the "Potential Payments Upon Termination or Change of Control" below.

#### Potential Payments Upon Termination or Change of Control

#### Agreements in Place Prior to the Arrangement

The employment agreements stipulate that in the event of the named executive officer's death or disability, we will pay all earned and accrued salary, bonus and vacation payments to the executive or the executive's estate. Additionally, Dr. Baum's employment agreement provides that he will be entitled to his annual bonus, prorated to the date of his death or incapacity.

The employment agreements provide that in the event of a termination without cause or resignation for good reason, we will pay:

any earned and accrued base salary, bonus and vacation pay;

base salary payments equal to 12 months of base salary (for Dr. Baum, payable in a lump sum cash payment and for Mr. Gergen, payable in equal monthly installments);

with respect to Dr. Baum, prorated target bonus payments (equal to 50% of the annual bonus target);

with respect to Dr. Baum, a target bonus payment equal to 50% of his annual target bonus;

with respect to Dr. Baum, continued vesting of all stock options for 12 months following termination; and

continued participation in the health, medical and life insurance programs for 12 months.

The employment agreements also provide for termination benefits in connection with a change of control. The following benefits are provided to Dr. Baum, in the event of his termination without cause or resignation for good reason within six months following a change of control or termination by Dr. Baum within three months following a change of control, and to Mr. Gergen in the event of termination without cause or resignation for good reason within twelve months following a change of control:

any earned and accrued base salary and vacation pay;

payments equal to 12 months of base salary and one times his annual target bonus (for Dr. Baum, payable in a lump sum if he resigns for good reason within three months following a change of control) or 24 months of base salary and two times his annual target bonus (for Dr. Baum, payable in a lump sum if he resigns for good reason or is terminated by us within six months following a change of control); and 18 months of base salary (for Dr. Gergen, payable in a lump sum);

with respect to Dr. Baum, prorated target bonus payments;

with respect to Dr. Baum, full vesting acceleration of all stock options; and

with respect to Dr. Baum, continued participation in the health, medical and life insurance programs for 12 months following his termination or resignation.

Under the terms of our Stock Option Plan and the 2013 Plan, options held by our executive officers may be subject to acceleration, termination or other treatment in connection with a change of control transaction or their termination of employment, as described in the section titled "Equity Plans" below.

#### Agreements in Place Upon and Following the Arrangement

Upon consummation of the Arrangement, the amended and restated employment agreements with Dr. Baum, Dr. Humphrey and Mr. Gergen replaced and superseded the terms of each of their employment agreements and clarified that the consummation of the Arrangement did not constitute a change of control for purposes of the prior employment agreements or the amended and restated employment agreements. Under the amended and restated employment agreements, all severance payments are conditioned upon the executive providing a release of claims against us.

The amended and restated employment agreements stipulate that in the event of the executive's death or disability, we will pay all earned and accrued salary, bonus and vacation payments to the executive or the executive's estate.

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The amended and restated employment agreements provide that in the event of a termination without cause or, in the case of Dr. Baum and Mr. Gergen, his resignation for good reason, we will pay:

any earned and accrued base salary, bonus and vacation pay;

a lump sum cash payment equal to 12 months of base salary;

with respect to Dr. Baum, 50% of his annual bonus prorated to his date of termination and 50% of his annual target bonus;

with respect to Dr. Baum, continued vesting of all stock options for 12 months following termination; and

with respect to Dr. Baum, payment of COBRA group health insurance premiums for up to 12 months.

The amended and restated employment agreements also provide for termination benefits in connection with a change of control. The following benefits are provided to (1) Dr. Baum, in the event of his termination without cause or resignation for good reason within twelve months following a change of control or termination by Dr. Baum for any reason within three months following a change of control; and (2) Mr. Gergen, in the event of his termination for good reason within twelve months following a change of control:

any earned and accrued base salary and vacation pay;

with respect to Dr. Baum, if he resigns for any reason within three months following a change of control, payments equal to 12 months of base salary and one times his annual target bonus or if he resigns for good reason or is terminated by us without cause within twelve months following a change of control, 24 months of base salary and two times his annual target bonus payable in a lump sum;

with respect to Mr. Gergen, 18 months base salary, payable in a lump sum;

with respect to Dr. Baum, full vesting acceleration of all stock options; and

with respect to Dr. Baum, payment of COBRA group health insurance premiums for up to 18 months.

#### **Equity Plans**

#### Stock Option Plan

Our Board of Directors and stockholders originally approved our Stock Option Plan in 1997 and approved various amendments, most recently in June 2012. As of December 31, 2012 we had 700,000 shares of common stock reserved for issuance under the Stock Option Plan. As of December 31, 2012, 2,555 shares of common stock had been issued under the Stock Option Plan and 559,815 shares were issuable under outstanding options granted under the Stock Option Plan.

As of June 30, 2013, we had 473,195 options outstanding under the Stock Option Plan. The 2013 Plan is a continuation of and successor to the Stock Option Plan and accordingly, no further grants will be made under the Stock Option Plan as of July 12, 2013.

Pursuant to the Stock Option Plan, options may be granted to our employees, officers, directors and consultants and is available to U.S. residents, and the term, exercise price, number of shares of common stock covered by each option, as well as the permitted frequency of the exercise of such options, is determined by the Board of Directors (or committee thereof) at the time the options are granted, in accordance with the criteria set out in the Stock Option Plan.

The exercise price of any option granted was based on our closing stock price as reported on the TSX at the end of the day prior to the option award date. In the event that there was no trading on the day prior to the option award date then the exercise price of the option award was determined by the volume-weighted average price on the five previous days on which the shares were traded. The period during which an option is exercisable and the vesting period of options were determined by the Board of Directors, in its sole discretion, at the time of granting the particular option award. The period during which an option is

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exercisable shall not, subject to the provisions of the Stock Option Plan, exceed 10 years from the date the option is granted. Since 2005, most options granted under the Stock Option Plan expire five years after the date the option is granted. However, if the term of an option expires during, or within ten business days after the expiration of, a blackout period (as that term is defined in the Stock Option Plan), then the term of such option or the unexercised portion thereof, shall be extended by ten business days after the expiration of the blackout period (the "Blackout Expiration Term"), provided that the Blackout Expiration Term will be reduced by the number of days between the date of the expiration of the term of the option and the end of the blackout period. The Board of Directors determined that effective January 1, 2012, stock options vest 20% on the date of award and then equally over four years, unless specifically stated otherwise. Previously options vested 25% on the date of award and then equally over three years instead of the previous term of five years. Under certain circumstances, including mergers, amalgamations and consolidations or in the event of an offer to purchase the shares of common stock, the exercise period of an option may be accelerated.

Options are not transferable and may be exercised by optionees while such optionees remain an employee, officer, director or consultant. If an optionee resigns his/her employment or if he/she ceases to be a director or consultant for any reason other than death, as the case may be, his/her non-vested options expire on the date termination while vested options expire 90 days after the date of his/her termination subject to the Board of Directors' right to alter any vesting period. If an optionee's employment, directorship or consulting agreement, as the case may be, is terminated by reason of death, his/her options will expire 180 days following the date of such termination subject to the Board of Directors' right to alter any vesting period. Upon an optionee's employment or a consultant's consultation agreement being terminated for just cause or resignation or termination at a time at which grounds for dismissal or termination for just cause exist, or upon an optionee being removed from office as a director, any option or the unexercised portion thereof granted to him/her shall terminate forthwith subject to the Board of Directors' right to alter any vesting period.

The Stock Option Plan provides optionees with an election for a cashless exercise of an optionee's vested and exercisable options. The number of shares of common stock to be acquired under a cashless exercise shall be equal to the quotient obtained when the difference between the volume-weighted average price of the shares of common stock on the five previous days on which the shares of common stock were traded (the "Market Price") and the exercise price of the options is divided by the Market Price, multiplied by the number of options exercised.

The Stock Option Plan provides that, upon the exercise of an option, the optionee shall make arrangements to our satisfaction regarding payment of any taxes of any kind required by law to be paid in connection with the exercise of the option. In order to satisfy our obligation, if any, to remit an amount to a taxation authority on account of the optionee's taxes in respect of the exercise, transfer or other disposition of an option, we have the right, at our sole discretion, to: (1) withhold amounts from any amounts owing to the optionee, whether under the Stock Option Plan or otherwise; (2) require the optionee to pay us the withholding tax amount as a condition of exercise of the option by an optionee; or (3) withhold from the shares of common stock otherwise deliverable to the optionee on exercise of the option such number of shares of common stock as have a market value not less than the withholding tax amount and cause such withheld shares of common stock to be sold on the optionee's behalf to fund the withholding tax amount, provided that any proceeds from such sale in excess of the withholding tax amount shall be promptly paid over to the optionee. Notwithstanding the foregoing, nothing precludes us and the optionee from agreeing to use a combination of the methods described above or some other method to fund the withholding tax amount.

Under the Stock Option Plan, the Board of Directors may, at any time, subject to regulatory approval, amend, suspend or terminate the Stock Option Plan in whole or in part. Without obtaining stockholder

approval, the Stock Option Plan may be amended by the Board of Directors for any purpose whatsoever, including, without limitation for the purpose of:

amendments of a "housekeeping" nature;

a change to the vesting provisions of an option;

a change to the termination provisions of an option or the Stock Option Plan which does not entail an extension beyond the original expiration date;

the addition of a cashless exercise feature payable in cash or securities; and

the addition of any form of financial assistance under the Stock Option Plan; provided, however, that no such amendment may:

increase the maximum number of shares of common stock issuable pursuant to the Stock Option Plan;

change the manner of determining the minimum option price;

alter the blackout expiration term;

reduce the option price per share for options granted to insiders under the Stock Option Plan;

extend the term of an option granted to insiders under the Stock Option Plan (subject to the blackout expiration term);

remove or exceed the insider participation limit under the Stock Option Plan;

amend the amending provision of the Stock Option Plan; or

without the consent of the optionee, adversely alter or impair any option previously granted to an optionee under the Stock Option Plan, without the consent of our stockholders, except to the extent required by law or by the regulations, rules, by-laws or policies of any regulatory authority or stock exchange.

In the event we propose to consolidate, merge, amalgamate, reorganize, be arranged or undergo an internal reorganization (other than with our wholly-owned subsidiary or to liquidate, dissolve or wind-up, or in the event an offer to purchase the shares of common stock or any part thereof shall be made to all holders of shares of common stock (hereinafter individually referred to as an "Event"), we shall have the right, upon written notice thereof to each optionee holding options under the Stock Option Plan, to permit the exercise of all such options within the 30-day period next following the date of such notice and to determine that upon the expiration of such 30-day period, all rights of optionees to such options or to exercise same (to the extent not theretofore exercised) shall ipso facto terminate and cease to have further force or effect whatsoever, provided, however, that if any Event results in a party or parties acting in concert obtaining control (as that term is defined in the Stock Option Plan) of us, we will give notice to each optionee of the acquisition of control and all unexercised options, including all options which have not

## Explanation of Responses:

yet vested, will immediately become exercisable at the option price for the 30-day period following the date of the Event, at the expiration of which period all unexercised options will be deemed to have vesting periods and vesting conditions originally applicable prior to such Event.

#### 2013 Equity Incentive Plan

In May 2013 our Board of Directors adopted the 2013 Plan. The 2013 Plan was approved by our stockholders in connection with the Arrangement. The 2013 Plan is a continuation of and successor to the Stock Option Plan and no further grants will be made under the Stock Option Plan.

*Stock Awards.* The 2013 Plan provides for the grant of incentive stock options, or ISOs, nonstatutory stock options, or NSOs, stock appreciation rights, restricted stock awards, restricted stock unit awards, performance-based stock awards, and other forms of equity compensation (collectively, stock awards), all of which may be granted to employees, including officers, non-employee directors and consultants of us and our affiliates. Additionally, the 2013 Plan provides for the grant of performance cash awards. ISOs may be granted only to employees. All other awards may be granted to employees, including officers, and to non-employee directors and consultants.

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*Share Reserve.* Initially, the aggregate number of shares of our common stock that may be issued pursuant to stock awards under the 2013 Plan is (1) 400,000 shares, plus (2) the number of shares remaining available for grant under our Stock Option Plan at the time the 2013 Plan became effective, plus (3) any shares subject to outstanding stock options or other stock awards that would have otherwise returned to our Stock Option Plan (such as upon the expiration or termination of a stock award prior to vesting). The maximum number of shares that may be issued upon the exercise of ISOs under the 2013 Plan is 1,097,444 shares.

No person may be granted stock awards covering more than 500,000 shares of our common stock under the 2013 Plan during any calendar year pursuant to stock options, stock appreciation rights and other stock awards whose value is determined by reference to an increase over an exercise or strike price of at least 100% of the fair market value on the date the stock award is granted. Additionally, no person may be granted in a calendar year a performance stock award covering more than 500,000 shares or a performance cash award having a maximum value in excess of \$1,000,000. Such limitations are designed to help assure that any deductions to which we would otherwise be entitled with respect to such awards will not be subject to the \$1,000,000 limitation on the income tax deductibility of compensation paid to any covered executive officer imposed by Section 162(m) of the Code.

If a stock award granted under the 2013 Plan expires or otherwise terminates without being exercised in full, or is settled in cash, the shares of our common stock not acquired pursuant to the stock award again will become available for subsequent issuance under the 2013 Plan. In addition, the following types of shares under the 2013 Plan may become available for the grant of new stock awards under the 2013 Plan: (1) shares that are forfeited to or repurchased by us prior to becoming fully vested; (2) shares withheld to satisfy income or employment withholding taxes; or (3) shares used to pay the exercise or purchase price of a stock award. Shares issued under the 2013 Plan may be previously unissued shares or reacquired shares bought by us on the open market. As of the date hereof, stock options to purchase an aggregate of 389,240 shares of common stock have been granted and no shares of our common stock have been issued under the 2013 Plan.

*Administration.* Our Board of Directors, or a duly authorized committee thereof, has the authority to administer the 2013 Plan. Our Board of Directors may also delegate to one or more of our officers the authority to (1) designate employees (other than other officers) to be recipients of certain stock awards, and (2) determine the number of shares of common stock to be subject to such stock awards. Subject to the terms of the 2013 Plan, our Board of Directors or the authorized committee, referred to herein as the plan administrator, determines recipients, dates of grant, the numbers and types of stock awards to be granted and the terms and conditions of the stock awards, including the period of their exercisability and vesting schedule applicable to a stock award. Subject to the limitations set forth below, the plan administrator will also determine the exercise price, strike price or purchase price of awards granted and the types of consideration to be paid for the award.

The plan administrator has the authority to modify outstanding awards under the 2013 Plan. Subject to the terms of the 2013 Plan, the plan administrator has the authority to reduce the exercise, purchase or strike price of any outstanding stock award, cancel any outstanding stock award in exchange for new stock awards, cash or other consideration, or take any other action that is treated as a repricing under U.S. generally accepted accounting principles, with the consent of any adversely affected participant.

*Stock Options.* Incentive and nonstatutory stock options are granted pursuant to stock option agreements adopted by the plan administrator. The plan administrator determines the exercise price for a stock option, within the terms and conditions of the 2013 Plan, provided that the exercise price of a stock option generally cannot be less than 100% of the fair market value of our common stock on the date of grant. Options granted under the 2013 Plan vest at the rate specified by the plan administrator.

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The plan administrator determines the term of stock options granted under the 2013 Plan, up to a maximum of ten years. Unless the terms of an option holder's stock option agreement provide otherwise, if an option holder's service relationship with us, or any of our affiliates, ceases for any reason other than disability, death or cause, the option holder may generally exercise any vested options for a period of three months following the cessation of service. The option term may be extended in the event that exercise of the option following such a termination of service is prohibited by applicable securities laws or our insider trading policy. If an option holder's service relationship with us or any of our affiliates ceases due to disability or death, or an option holder dies within a certain period following cessation of service, the option holder or a beneficiary may generally exercise any vested options for a period of 42 months in the event of disability and 18 months in the event of death. In the event of a termination for cause, options generally terminate immediately upon the termination of the individual for cause. In no event may an option be exercised beyond the expiration of its term.

Acceptable consideration for the purchase of common stock issued upon the exercise of a stock option will be determined by the plan administrator and may include (1) cash, check, bank draft or money order, (2) a broker-assisted cashless exercise, (3) the tender of shares of our common stock previously owned by the option holder, (4) a net exercise of the option if it is an NSO, and (5) other legal consideration approved by the plan administrator.

Unless the plan administrator provides otherwise, options generally are not transferable except by will, the laws of descent and distribution, or pursuant to a domestic relations order. An option holder may designate a beneficiary, however, who may exercise the option following the option holder's death.

*Tax Limitations On Incentive Stock Options.* The aggregate fair market value, determined at the time of grant, of our common stock with respect to ISOs that are exercisable for the first time by an option holder during any calendar year under all of our stock plans may not exceed \$100,000. Options or portions thereof that exceed such limit will generally be treated as NSOs. No ISO may be granted to any person who, at the time of the grant, owns or is deemed to own stock possessing more than 10% of our total combined voting power or that of any of our affiliates unless (1) the option exercise price is at least 110% of the fair market value of the stock subject to the option on the date of grant, and (2) the term of the ISO does not exceed five years from the date of grant.

*Restricted Stock Awards.* Restricted stock awards are granted pursuant to restricted stock award agreements adopted by the plan administrator. Restricted stock awards may be granted in consideration for (1) cash, check, bank draft or money order, (2) services rendered to us or our affiliates, or (3) any other form of legal consideration. Common stock acquired under a restricted stock award may, but need not, be subject to a share repurchase option in our favor in accordance with a vesting schedule to be determined by the plan administrator. Rights to acquire shares under a restricted stock award may be transferred only upon such terms and conditions as set by the plan administrator. Except as otherwise provided in the applicable award agreement, restricted stock unit awards that have not vested will be forfeited upon the participant's cessation of continuous service for any reason.

*Restricted Stock Unit Awards.* Restricted stock unit awards are granted pursuant to restricted stock unit award agreements adopted by the plan administrator. Restricted stock unit awards may be granted in consideration for any form of legal consideration. A restricted stock unit award may be settled by cash, delivery of stock, a combination of cash and stock as deemed appropriate by the plan administrator, or in any other form of consideration set forth in the restricted stock unit award agreement. Additionally, dividend equivalents may be credited in respect of shares covered by a restricted stock unit award. Except as otherwise provided in the applicable award agreement, restricted stock units that have not vested will be forfeited upon the participant's cessation of continuous service for any reason.

*Stock Appreciation Rights.* Stock appreciation rights are granted pursuant to stock appreciation grant agreements adopted by the plan administrator. The plan administrator determines the strike price for a

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stock appreciation right, which generally cannot be less than 100% of the fair market value of our common stock on the date of grant. Upon the exercise of a stock appreciation right, we will pay the participant an amount equal to the product of (1) the excess of the per share fair market value of our common stock on the date of exercise over the strike price, multiplied by (2) the number of shares of common stock with respect to which the stock appreciation right is exercised. A stock appreciation right granted under the 2013 Plan vests at the rate specified in the stock appreciation right agreement as determined by the plan administrator.

The plan administrator determines the term of stock appreciation rights granted under the 2013 Plan, up to a maximum of ten years. Unless the terms of a participant's stock appreciation right agreement provides otherwise, if a participant's service relationship with us or any of our affiliates ceases for any reason other than cause, disability or death, the participant may generally exercise any vested stock appreciation right for a period of three months following the cessation of service. The stock appreciation right term may be further extended in the event that exercise of the stock appreciation right following such a termination of service is prohibited by applicable securities laws. If a participant's service relationship with us, or any of our affiliates, ceases due to disability or death, or a participant dies within a certain period following cessation of service, the participant or a beneficiary may generally exercise any vested stock appreciation right for a period of 12 months in the event of death. In the event of a termination for cause, stock appreciation rights generally terminate immediately upon the occurrence of the event giving rise to the termination of the individual for cause. In no event may a stock appreciation right be exercised beyond the expiration of its term.

*Performance Awards.* The 2013 Plan permits the grant of performance-based stock and cash awards that may qualify as performance-based compensation that is not subject to the \$1,000,000 limitation on the income tax deductibility of compensation paid to a covered executive officer imposed by Section 162(m) of the Code. To help assure that the compensation attributable to performance-based awards will so qualify, our Compensation Committee can structure such awards so that stock or cash will be issued or paid pursuant to such award only after the achievement of certain pre-established performance goals during a designated performance period.

The performance goals that may be selected include one or more of the following: (1) earnings (including earnings per share and net earnings); (2) earnings before interest, taxes and depreciation; (3) earnings before interest, taxes, depreciation and amortization; (4) total stockholder return; (5) return on equity or average stockholders' equity; (6) return on assets, investment, or capital employed; (7) stock price; (8) margin (including gross margin); (9) income (before or after taxes); (10) operating income; (11) operating income after taxes; (12) pre-tax profit; (13) operating cash flow; (14) sales or revenue targets; (15) increases in revenue or product revenue; (16) expenses and cost reduction goals; (17) improvement in or attainment of working capital levels; (18) economic value added (or an equivalent metric); (19) market share; (20) cash flow; (21) cash flow per share; (22) share price performance; (23) debt reduction; (24) implementation or completion of projects or processes; (25) customer satisfaction; (26) stockholders' equity; (27) capital expenditures; (28) debt levels; (29) operating profit or net operating profit; (30) workforce diversity; (31) growth of net income or operating income; (32) billings; and (33) to the extent that an award is not intended to comply with Section 162(m) of the Code, other measures of performance selected by our Board of Directors.

The performance goals may be based on a company-wide basis, with respect to one or more business units, divisions, affiliates, or business segments, and in either absolute terms or relative to the performance of one or more comparable companies or the performance of one or more relevant indices. Unless specified otherwise (1) in the award agreement at the time the award is granted or (2) in such other document setting forth the performance goals at the time the goals are established, we will appropriately make adjustments in the method of calculating the attainment of performance goals as follows: (i) to exclude restructuring and/or other nonrecurring charges; (ii) to exclude exchange rate effects; (iii) to exclude the effects of changes to

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generally accepted accounting principles; (iv) to exclude the effects of any statutory adjustments to corporate tax rates; and (v) to exclude the effects of any "extraordinary items" as determined under U.S. generally accepted accounting principles. In addition, we retain the discretion to reduce or eliminate the compensation or economic benefit due upon attainment of the goals. The performance goals may differ from participant to participant and from award to award.

*Other Stock Awards.* The plan administrator may grant other awards based in whole or in part by reference to our common stock. The plan administrator will set the number of shares under the stock award and all other terms and conditions of such awards.

*Changes to Capital Structure.* In the event that there is a specified type of change in our capital structure, such as a stock split or recapitalization, appropriate adjustments will be made to (1) the class and maximum number of shares reserved for issuance under the 2013 Plan, (2) the class and maximum number of shares that may be issued upon the exercise of ISOs, (3) the class and maximum number of shares subject to stock awards that can be granted in a calendar year (as established under the 2013 Plan pursuant to Section 162(m) of the Code) and (4) the class and number of shares and exercise price, strike price, or purchase price, if applicable, of all outstanding stock awards.

*Corporate Transactions.* In the event of certain specified significant corporate transactions, the plan administrator has the discretion to take any of the following actions with respect to stock awards:

arrange for the assumption, continuation or substitution of a stock award by a surviving or acquiring entity or parent company;

arrange for the assignment of any reacquisition or repurchase rights held by us to the surviving or acquiring entity or parent company;

accelerate the vesting of the stock award and provide for its termination prior to the effective time of the corporate transaction;

arrange for the lapse of any reacquisition or repurchase right held by us;

cancel or arrange for the cancellation of the stock award in exchange for such cash consideration, if any, as our Board of Directors may deem appropriate; or

make a payment equal to the excess of (1) the value of the property the participant would have received upon exercise of the stock award over (2) the exercise price otherwise payable in connection with the stock award.

Our plan administrator is not obligated to treat all stock awards, even those that are of the same type, in the same manner.

Under the 2013 Plan, a corporate transaction is generally the consummation of (1) a sale or other disposition of all or substantially all of our consolidated assets, (2) a sale or other disposition of at least 90% of our outstanding securities, (3) a merger, consolidation or similar transaction following which we are not the surviving corporation, or (4) a merger, consolidation or similar transaction following which we are the surviving corporation but the shares of our common stock outstanding immediately prior to such transaction are converted or exchanged into other property by virtue of the transaction.

*Change of Control.* The plan administrator may provide, in an individual award agreement or in any other written agreement between a participant and us that the stock award will be subject to additional acceleration of vesting and exercisability in the event of a change of control. Under the 2013 Plan, a change of control is generally (1) the acquisition by a person or entity of more than 50% of our combined voting power other than by merger, consolidation or similar transaction; (2) a consummated merger, consolidation or similar transaction immediately after which our stockholders cease to own more than 50% of the combined voting power of the surviving entity; (3) a consummated sale, lease or exclusive license or other disposition of all or substantially of our consolidated assets; or (4) when a majority of the Board of

Directors becomes comprised of individuals whose nomination, appointment, or election was not approved by a majority of the Board of Directors members or their approved successors.

Amendment and Termination. Our Board of Directors has the authority to amend, suspend, or terminate the 2013 Plan, provided that such action does not materially impair the existing rights of any participant without such participant's written consent. No ISOs may be granted after the tenth anniversary of the date our Board of Directors adopted the 2013 Plan.

#### 2013 Employee Stock Purchase Plan

In May 2013 our Board of Directors adopted the ESPP. The ESPP was approved by our stockholders in connection with the Arrangement. The purpose of the ESPP is to retain the services of new employees and secure the services of new and existing employees while providing incentives for such individuals to exert maximum efforts toward our success and that of our affiliates.

*Share Reserve.* The ESPP authorizes the issuance of 300,000 shares of our common stock pursuant to purchase rights granted to our employees or to employees of any of our designated affiliates. The ESPP is intended to qualify as an "employee stock purchase plan" within the meaning of Section 423 of the Code. As of the date hereof, no shares of our common stock have been purchased under the ESPP.

*Administration.* Our Board of Directors, or a duly authorized committee thereof, has the authority to administer the ESPP. The ESPP is implemented through a series of offerings of purchase rights to eligible employees. Under the ESPP, we may specify offerings with durations of not more than 27 months, and may specify shorter purchase periods within each offering. Each offering will have one or more purchase dates on which shares of our common stock will be purchased for employees participating in the offering. An offering may be terminated under certain circumstances.

*Payroll Deductions.* Generally, all regular employees, including executive officers, employed by us or by any of our designated affiliates, may participate in the ESPP and may contribute, normally through payroll deductions, up to 15% of their earnings for the purchase of our common stock under the ESPP. Unless otherwise determined by our Board of Directors, common stock will be purchased for accounts of employees participating in the ESPP at a price per share equal to the lower of (1) 85% of the fair market value of a share of our common stock on the first date of an offering or (2) 85% of the fair market value of a share of our common stock on the date of purchase.

*Limitations.* Employees may have to satisfy one or more of the following service requirements before participating in the ESPP, as determined by our Board of Directors: (1) customarily employed for more than 20 hours per week, (2) customarily employed for more than five months per calendar year or (3) continuous employment with us or one of our affiliates for a period of time (not to exceed two years). No employee may purchase shares under the ESPP at a rate in excess of \$25,000 worth of our common stock based on the fair market value per share of our common stock at the beginning of an offering for each year such a purchase right is outstanding. Finally, no employee will be eligible for the grant of any purchase rights under the ESPP if immediately after such rights are granted, such employee has voting power over 5% or more of our outstanding capital stock measured by vote or value pursuant to Section 424(d) of the Code.

*Changes to Capital Structure*. In the event that there occurs a change in our capital structure through such actions as a stock split, merger, consolidation, reorganization, recapitalization, reincorporation, stock dividend, dividend in property other than cash, large nonrecurring cash dividend, liquidating dividend, combination of shares, exchange of shares, change in corporate structure or similar transaction, the Board of Directors will make appropriate adjustments to (1) the number of shares reserved under the ESPP and (2) the number of shares and purchase price of all outstanding purchase rights.

*Corporate Transactions.* In the event of certain significant corporate transactions, including: (1) a sale of all our assets, (2) the sale or disposition of 90% of our outstanding securities, (3) the consummation of a merger or consolidation where we do not survive the transaction, and (4) the consummation of a merger or



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consolidation where we do survive the transaction but the shares of our common stock outstanding immediately prior to such transaction are converted or exchanged into other property by virtue of the transaction, any then-outstanding rights to purchase our stock under the ESPP may be assumed, continued or substituted for by any surviving or acquiring entity (or its parent company). If the surviving or acquiring entity (or its parent company) elects not to assume, continue or substitute for such purchase rights, then the participants' accumulated payroll contributions will be used to purchase shares of our common stock within ten business days prior to such corporate transaction, and such purchase rights will terminate immediately.

*Plan Amendments, Termination.* Our Board of Directors has the authority to amend or terminate our ESPP, provided that except in certain circumstances any such amendment or termination may not materially impair any outstanding purchase rights without the holder's consent. We will obtain stockholder approval of any amendment to our ESPP as required by applicable law or listing requirements.

#### **Non-Executive Director Compensation**

The Compensation Committee reviews and recommends the compensation of non-employee directors to the Board of Directors on an annual basis. The following table summarizes the compensation earned by or paid to each of the non-employee directors in 2012:

	FEES EARNED OR PAID			
	IN	OPTION	ALL OTHER	
NAME	CASH(2)	AWARDS(1)(10)	COMPENSATION(2)	TOTAL(2)
Martin Godbout, O.C., Ph.D. <sup>(3)</sup>	\$ 77,684	\$ 131,572	\$ 19,919(4)	\$ 229,175
Peter Thompson, M.D.	43,000	65,786		108,786
Henry J. Fuchs, M.D. <sup>(5)</sup>	37,643	88,991		126,634
Margaret Mulligan <sup>(3)(6)</sup>	39,966	89,011		128,977
Rodney Lappe, Ph.D. <sup>(7)</sup>	20,000	83,259		103,259
Louis Lacasse <sup>(3)(9)</sup>	47,308	65,786		113,094
Colin R. Mallet <sup>(3)(9)</sup>	48,801	65,786		114,588
David J. Drutz, M.D. <sup>(8)</sup>	24,000			24,000

(1)

Each newly appointed non-executive director received options upon his/her appointment. Directors were also granted an annual stock option award on July 17, 2012. In accordance with SEC rules, this column reflects the aggregate grant date fair value of the option awards granted during 2012 computed in accordance with Financial Accounting Standard Board Accounting Standards Codification Topic 718 for stock-based compensation transactions (ASC 718). Assumptions used in the calculation of these amounts are included in Note 13 to our consolidated financial statements appearing elsewhere in this prospectus. These amounts do not reflect the actual economic value that will be realized by the non-executive directors upon the vesting of the stock options, the exercise of the stock options, or the sale of the common stock underlying such stock options. The value of all option awards in the table above was originally calculated in Canadian dollars and was converted to the U.S. dollar amount in the table above using the average monthly U.S. dollar per Canadian dollar conversion rate from the Bank of Canada for the month in which the grant date occurred, which was 0.9869, 1.0035, 1.0062, 0.9727 and 0.9863 for January, February, March, June and July 2012 grant dates, respectively. The vesting period was changed effective January 1, 2012 to 20% on the date of the award and then 20% on each of the next four anniversary dates.

## (2)

Payments to directors resident in the United States are paid in U.S. dollars while payments to Canadian directors are paid in Canadian dollars. The conversion rate used to convert Canadian payments to U.S. dollars in the table above was the weekly average U.S. dollar per Canadian dollar conversion rate from the Bank of

Canada for each week in which a payment was made.

# (3) Dr. Godbout, Ms. Mulligan and Messrs. Lacasse and Mallet were paid in Canadian dollars. (4) This amount represents an additional discretionary amount paid to Dr. Godbout in recognition for his efforts in connection with the transition period relating to the change of our Chief Executive Officer. (5) Dr. Fuchs was appointed to the Board of Directors in February 2012. (6) Ms. Mulligan was appointed to the Board of Directors in March 2012. Ms. Mulligan did not stand for re-election and her mandate as a director terminated in June 2013.

Dr. Lappe was elected to the Board of Directors at our annual meeting in June 2012.

## (8)

(7)

Dr. Drutz did not stand for re-election and his mandate as a director terminated in June 2012.

## (9)

Messrs. Lacasse and Mallet resigned from the Board of Directors in November 2012.

## (10)

NAME

The following table lists the aggregate number of outstanding stock options (whether vested or unvested) held by each of our non-employee directors as of December 31, 2012:

# TOTAL NUMBER OF SHARES UNDERLYING OUTSTANDING STOCK OPTIONS (#) 22,721 10,953

Martin Godbout, O.C., Ph.D.	22,721
Peter Thompson, M.D.	10,953
Henry J. Fuchs, M.D.	8,953
Margaret Mulligan	8,953
Rodney Lappe, Ph.D.	8,953
Colin R. Mallet	4,516
Louis Lacasse	4,401
David J. Drutz, M.D.	
Total	69,450

The following table summarizes the annual compensation for non-executive directors in 2012:

CASH COMPENSATION		STOCK-BASED COMPENSATION	
Board of Directors annual retainer	\$ 40,000	Number of shares underlying stock option	
Incremental annual retainer for the Chairman	\$ 25,000	granted upon joining the Board	2,000
Committee Chair annual retainer		Number of shares underlying annual stock	
Audit	\$ 10,000	options awarded in 2012	
Compensation	\$ 5,000	Directors	6,953
Corporate Governance and Nominating	\$ 5,000	Incremental to the Chairman	6,953
Committee member annual retainer			
Audit	\$ 5,000		
Compensation	\$ 3,000		
Corporate Governance and Nominating	\$ 3,000		

The above cash retainers are paid in U.S. dollars for U.S.-based directors and in Canadian dollars for Canadian-based directors. Director's fees are prorated to the date the director is appointed or elected. In addition, directors are reimbursed for all reasonable and documented travel-related expenses incurred by them in order to attend Board of Directors and committee meetings, subject to our travel policy.

## Limitation of Liability and Indemnification

Our amended and restated certificate of incorporation limits the liability of directors to the maximum extent permitted by Delaware law. Delaware law provides that directors of a corporation will not be personally liable for monetary damages for breach of their fiduciary duties as directors, except for liability for any:

breach of their duty of loyalty to the corporation or its stockholders;

act or omission not in good faith or that involves intentional misconduct or a knowing violation of law;

unlawful payments of dividends or unlawful stock repurchases or redemptions as provided in Section 174 of the Delaware General Corporation Law; or

transaction from which the directors derived an improper personal benefit.

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Our amended and restated certificate of incorporation does not eliminate a director's duty of care and, in appropriate circumstances, equitable remedies, such as injunctive or other forms of non-monetary relief, remain available under Delaware law. These limitations also do not affect a director's responsibilities under any other laws, such as the federal securities laws or other state or federal laws. Our bylaws, provide that we will indemnify our directors and executive officers, and may indemnify other officers, employees and other agents, to the fullest extent permitted by law. Our bylaws also provide that we are obligated to advance expenses incurred by a director or officer in advance of the final disposition of any action or proceeding and also permit us to secure insurance on behalf of any officer, director, employee or other agent for any liability arising out of his or her actions in connection with their services to us, regardless of whether our amended and restated bylaws permit such indemnification. We have obtained a directors' liability insurance policy.

We have entered, and intend to continue to enter, into separate indemnification agreements with our directors and executive officers, in addition to the indemnification provided for in our bylaws. These agreements, among other things, require us to indemnify our directors and executive officers for certain expenses, including attorneys' fees, judgments, fines and settlement amounts incurred by a director or executive officer in any action or proceeding arising out of their services as one of our directors or executive officers, or any of our subsidiaries or any other company or enterprise to which the person provides services at our request. We believe that these bylaw provisions and indemnification agreements are necessary to attract and retain qualified persons as directors and officers.

The limitation of liability and indemnification provisions in our amended and restated certificate of incorporation and bylaws may discourage stockholders from bringing a lawsuit against directors for breach of their fiduciary duties. They may also reduce the likelihood of derivative litigation against directors and officers, even though an action, if successful, might benefit us and our stockholders. A stockholder's investment may be harmed to the extent we pay the costs of settlement and damage awards against directors and officers pursuant to these indemnification provisions.

At present, there is no pending litigation or proceeding involving any of our directors or executive officers as to which indemnification is required or permitted, and we are not aware of any threatened litigation or proceeding that may result in a claim for indemnification.

## CERTAIN RELATIONSHIPS AND RELATED PARTY TRANSACTIONS

The following is a description of transactions since January 1, 2011 to which we have been a party, in which the amount involved exceeded or will exceed the lesser of \$120,000 or 1% of the average of our total assets at year end for the last two years, and in which any of our executive officers, directors or holders of more than 5% of our common stock, or an affiliate or immediate family member thereof, had or will have a direct or indirect material interest, other than compensation, termination and change of control arrangements, which are described under "Executive and Director Compensation."

#### **Director Affiliations With Our Principal Stockholders**

Some of our directors are affiliated with our principal stockholders as indicated in the table below:

**DIRECTOR** Rodney W. Lappe, Ph.D. Peter Thompson, M.D. AFFILIATION Tavistock Life Sciences OrbiMed Advisors LLC

#### **Stock Issuances**

#### 2012 Private Placements

In November 2012, we entered into a securities purchase agreement, or the 2012 Securities Purchase Agreement, pursuant to which we sold 3,593,819 units at a subscription price per unit of CND\$7.25 (or US\$6.99, as converted as of October 15, 2013), with each unit consisting of one share of common stock and thirty one-hundredths (0.30) of a warrant to purchase a share of common stock, exercisable until November 21, 2017 at an exercise price of CND\$8.70 (or US\$8.39, as converted as of October 15, 2013) (being 120% of the subscription price), for net proceeds of \$24.8 million. The issuance costs of the units amounted to \$1.3 million.

		SHARES OF COMMON		Р	URCHASE
NAME	UNITS	STOCK	WARRANTS		PRICE
Baker Bros. Advisors, L.L.C.	934,218	934,218	280,265	US\$	6,801,107
Tavistock Life Sciences	893,222	893,222	267,966	US\$	6,502,656
RA Capital Healthcare Fund, L.P.	551,724	551,724	165,517	US\$	4,016,551
Tang Capital Partners, L.P.	413,793	413,793	124,138	US\$	3,012,413
OrbiMed Advisors LLC	344,827	344,827	103,448	US\$	2,510,341
BVF Investments L.L.C.	275,862	275,862	82,758	US\$	2,008,275

Each of Baker Brothers, Tavistock and Tang Capital agreed that it will not exercise any portion of its warrants acquired under the 2012 Securities Purchase Agreement to the extent that, after giving effect to such exercise, it would beneficially own in excess of 19.9%, in the case of Baker Brothers and Tavistock, or 9.99%, in the case of Tang Capital, of the shares of our common stock, except in certain limited circumstances. Pursuant to the 2012 Securities Purchase Agreement, we granted to Baker Brothers and Tavistock, for so long as each owns at least 10% of our issued and outstanding capital stock on a partially diluted basis (assuming only the exercise of any convertible securities or rights to acquire shares of common stock of each of Baker Brothers and Tavistock), the right to nominate a member to our Board of Directors and the right to appoint an observer to our Board of Directors. For more information, see "Description of Capital Stock Board Observer and Nomination Rights."

### 2011 Private Placement

In April 2011, we entered into a securities purchase agreement, or the 2011 Securities Purchase Agreement, pursuant to which we sold 5,549,895 units at a subscription price per unit of CND\$6.22 (or US\$6.00, as converted as of October 15, 2013), with each unit consisting of one share of common stock and thirty one-hundredths (0.30) of a warrant to purchase a share of common stock, exercisable until April 4, 2016 at an exercise price of CND\$7.46 (or US\$7.19, as converted as of October 15, 2013) (being 120% of the subscription price), for net proceeds of \$33.7 million. This includes the conversion of convertible debentures that were issued by us in March 2011 to affiliates of Baker Brothers and Tavistock, for each to acquire 61,561 units for \$783,869. The issuance costs of the units amounted to \$2.0 million.

		SHARES OF COMMON		Р	URCHASE
NAME	UNITS	STOCK	WARRANTS		PRICE
Baker Bros. Advisors, L.L.C.	1,045,856	1,045,856	313,757	US\$	6,714,396
Tavistock Life Sciences	1,045,856	1,045,856	313,757	US\$	6,714,396
OrbiMed Advisors LLC	804,505	804,505	241,351	US\$	5,164,922
Tang Capital Partners, L.P.	804,505	804,505	241,351	US\$	5,164,922
QVT Fund, L.P.	804,505	804,505	241,351	US\$	5,164,922
BVF Investments L.L.C.	321,802	321,802	96,540	US\$	2,065,969

Each of Baker Brothers, Tavistock, OrbiMed, Tang Capital, QVT Fund and BVF Fund has agreed that it will not exercise any portion of its warrants acquired under the 2011 Securities Purchase Agreement to the extent that, after giving effect to such exercise, it would beneficially own in excess of 19.9%, in the case of Baker Brothers, Tavistock and OrbiMed, or 9.99%, in the case of Tang Capital, QVT or BVF, of our shares of common stock, except in certain limited circumstances. Pursuant to the 2011 Securities Purchase Agreement, we granted to Baker Brothers and Tavistock, for a period of two years following the closing of the transaction, the right to nominate a member to our Board of Directors and the right to appoint an observer to our Board of Directors. These rights, however, were superseded by the right to appoint an observer to our Board of Directors granted to Baker Brothers and Tavistock in the 2012 private placement described above.

#### Stock Options Granted to Executive Officers and Directors

We have granted stock options to our executive officers and directors, as more fully described in "Executive Compensation."

#### **Participation in Offering**

Certain of our existing stockholders, including certain affiliates of Tavistock and Baker Brothers, have indicated an interest in purchasing up to an aggregate of 1,207,143 shares of our common stock offered by us in this offering at the public offering price. However, because indications of interest are not binding agreements or commitments to purchase, the underwriters may determine to sell more, fewer or no shares in this offering to any of these stockholders, or any of these stockholders may determine to purchase more, fewer or no shares in this offering.

#### **Indemnification Agreements**

We have entered, and intend to continue to enter, into separate indemnification agreements with each of our directors and executive officers, as described in "Executive Compensation Limitation of Liability and Indemnification."

#### **Employment Arrangements**

We currently maintain written employment agreements with several of our named executive officers, as described in "Executive Compensation Employment Agreements."

#### Policies and Procedures for Transactions with Related Persons

We have adopted a written related-person transactions policy that sets forth our policies and procedures regarding the identification, review, consideration, approval and oversight of "related-person transactions." For purposes of our policy only, a "related-person transaction" is a past, present or future transaction, arrangement or relationship (or any series of similar transactions, arrangements or relationships) in which we and any "related person" are, were or will be participants involving an amount that exceeds \$120,000.

Transactions involving compensation for services provided to us by an employee, consultant or director will not be considered related-person transactions under this policy. A "related person," as determined since the beginning of our last fiscal year, is any executive officer, director or a holder of more than five percent of our common stock, including any of their immediate family members and any entity owned or controlled by such persons.

The policy imposes an affirmative duty upon each director and executive officer to identify, and we will request that significant stockholders identify, any transaction involving them, their affiliates or immediate family members that may be considered a related party transaction before such person engages in the transaction. Under the policy, where a transaction has been identified as a related-person transaction, management must present information regarding the proposed related-person transaction to our Audit Committee (or, where review by our Audit Committee would be inappropriate, to another independent body of our Board of Directors) for review. The presentation must include a description of, among other things, the material facts, the direct and indirect interests of the related persons, the benefits of the transaction to us and whether any alternative transactions are available. In considering related-person transactions, our Audit Committee or other independent body of our Board of Directors including, but not limited to:

the risks, costs and benefits to us of the transaction;

the impact on a director's independence in the event the related person is a director, immediate family member of a director or an entity with which a director is affiliated;

the terms of the transaction;

the availability of other sources for comparable services or products; and

the terms available to or from, as the case may be, unrelated third parties or to or from our employees generally.

In the event a director has an interest in the proposed transaction, the director must recuse himself or herself from the deliberations and approval. Our policy requires that, in reviewing a related party transaction, our Audit Committee must consider, in light of known circumstances, and determine in the good faith exercise of its discretion whether the transaction is in, or is not inconsistent with, the best interests of us and our stockholders.

#### **Compensation Committee Interlocks And Insider Participation**

None of our current or former executive officers serves as a member of our Compensation Committee. None of our officers serves, or has served during the last completed fiscal year on the board of directors or compensation committee, or other committee serving an equivalent function, of any other entity that has one or more of its executive officers serving as a member of our Board of Directors or our Compensation Committee.

## PRINCIPAL STOCKHOLDERS

The following table sets forth information regarding beneficial ownership of our capital stock as of August 31, 2013 by:

each person, or group of affiliated persons, known by us to beneficially own more than 5% of our common stock;

each of our directors;

each of our named executive officers; and

all of our directors and current executive officers as a group.

We have determined beneficial ownership in accordance with the rules of the SEC. Except as indicated by the footnotes below, we believe, based on the information available to us, that the persons and entities named in the table below have sole voting and investment power with respect to all shares of common stock that they beneficially own, subject to applicable community property laws.

Applicable percentage ownership prior to the offering is based on 9,957,725 shares of common stock outstanding at August 31, 2013. In computing the number of shares of common stock beneficially owned by a person and the percentage ownership of that person, we deemed to be outstanding all shares of common stock subject to options, warrants or other convertible securities held by that person or entity that are currently exercisable or will be exercisable within 60 days of August 31, 2013. We did not deem these shares outstanding, however, for the purpose of computing the percentage ownership of any other person. Except as otherwise noted below, the address for each person or entity is c/o Mirati Therapeutics, Inc., 9363 Towne Centre Drive, Suite 200, San Diego, California 92121.

Certain of our existing stockholders, including certain affiliates of Tavistock and Baker Brothers, have indicated an interest in purchasing up to an aggregate of 1,207,143 shares of our common stock offered by us in this offering at the public offering price. However, because indications of interest are not binding agreements or commitments to purchase, the underwriters may determine to sell more, fewer or no shares in this offering to any of these stockholders, or any of these stockholders may determine to purchase more, fewer or no shares in this offering. The information set forth in the table below does not reflect any potential purchase of any shares in this offering by such stockholders.

Name and Address of Beneficial Owner	Shares Beneficially Owned Before Offering	Percentage of Total Voting Power Before Offering	Percentage of Total Voting Power After Offering
5% Stockholders:			
Baker Bros. Advisors, L.L.C. <sup>(1)</sup>	1,980,074	19.9%	18.7%
Tavistock Life Sciences <sup>(2)</sup>	1,980,074	19.9%	18.6%
OrbiMed Private Investments IV, L.P. <sup>(3)</sup>	1,494,131	14.5%	11.0%
Tang Capital Partners, L.P. <sup>(4)</sup>	1,218,298	12.2%	10.0%
QVT Fund, L.P. <sup>(5)</sup>	834,328	8.2%	6.2%
BVF Investments, L.L.C. <sup>(6)</sup>	773,398	7.6%	5.8%
RA Capital Healthcare Fund, L.P. <sup>(7)</sup>	717,239	7.1%	5.4%
Directors and Named Executive Officers:			
Martin Godbout, O.C., Ph.D. <sup>(8)</sup>	14,433	*	*
Henry Fuchs, M.D. <sup>(9)</sup>	3,582	*	*
Charles M. Baum, M.D., Ph.D. <sup>(10)</sup>	79,600	*	*
Peter Thompson, M.D. <sup>(11)</sup>	5,782	*	*
Craig Johnson			
Rodney Lappe, Ph.D. <sup>(12)</sup>	3,582	*	*
Charles Grubsztajn <sup>(13)</sup>	1,800	*	*
Rachel Humphrey, M.D. <sup>(14)</sup>	33,066	*	*
Jeffrey M. Besterman <sup>(15)</sup>	670	*	*
All executive officers and directors as a group <sup>(16)</sup>	172,445	1.7%	1.3%

\*

Represents beneficial ownership of less than one percent of our outstanding shares of common stock.

## (1)

Includes 1,866,932 shares of common stock held by Baker Brother Sciences, L.P., 81,555 shares of common stock held by 667, L.P. and 31,587 shares of common stock held by 14159, L.P. Excludes, in the case of shares beneficially owned before the offering and for purposes of calculating the percentage of total voting power before the offering, 559,805 shares of common stock subject to warrants that are exercisable within 60 days of August 31, 2013 by Baker Brother Sciences, L.P., 24,467 shares of common stock subject to warrants that are exercisable within 60 days of August 31, 2013 by Baker Brother Sciences, L.P., 24,467 shares of common stock subject to warrants that are exercisable within 60 days of August 31, 2013 by 667, L.P., and 9,476 shares of common stock subject to warrants that are exercisable within 60 days of August 31, 2013 by 14159, L.P, and 275 shares of common stock subject to warrants that are exercisable within 60 days of August 31, 2013 by Baker Bros. Investments II, L.P. The warrants are not exercisable to the extent that any such exercise would increase such stockholders' aggregate ownership percentage in excess of 19.9% of our outstanding common stock, except in limited circumstances. Baker Bros. Investments II, L.P. and may be deemed to beneficially own Baker Brother Life Sciences, L.P.'s, 667, L.P.'s, 14159, L.P.'s, and Baker Bros. Investments II, L.P.'s shares of common stock and shares subject to warrants that are exercisable within 60 days of August 31, 2013. The address for Baker Bros. Advisors, L.L.C. is 667 Madison Avenue, 21st Floor, New York, NY 10065.

## (2)

Includes 1,590,733 shares of common stock held by Boxer Capital, L.L.C. and 389,341 shares of common stock held by MVA Investors, L.L.C. Excludes, in the case of shares beneficially owned before the offering and for purposes of calculating the percentage of total voting power before the offering, 462,662 shares subject to warrants that are exercisable within 60 days of August 31, 2013 by Boxer Capital, L.L.C and

119,060 shares subject to warrants that are exercisable within 60 days of August 31, 2013 by MVA Investors, L.L.C. The warrants are not exercisable to the extent that any such exercise would increase such aggregate ownership percentage in excess of 19.9% of our outstanding common stock, except in limited circumstances. Tavistock Life Sciences is the investment manager of Boxer Capital, L.L.C. and MVA Investors, L.L.C. and may be deemed to beneficially own Boxer Capital, L.L.C.'s and MVA Investors, L.L.C.'s shares of common stock and shares subject to warrants that are exercisable within 60 days of August 31, 2013. The address for Tavistock Life Sciences is 445 Marine View Avenue, Suite 100, Del Mar, CA 92014.

(3)

Includes 1,149,332 shares of common stock and 344,799 shares subject to warrants that are exercisable within 60 days of August 31, 2013. The warrants are not exercisable to the extent that any such exercise would increase the stockholder's ownership percentage in excess of 19.9% of our outstanding common stock, except in limited circumstances. OrbiMed Capital GP IV L.L.C. is the sole general partner of OrbiMed Private Investments IV, L.P. and as such may be deemed to indirectly beneficially own the shares held by OrbiMed Private Investments IV, L.P. OrbiMed Advisors L.L.C. pursuant to its authority as the sole managing member of OrbiMed Capital GP IV L.L.C. may be deemed to indirectly beneficially own the shares held by OrbiMed Private Investments IV, L.P. Samuel D. Isaly is the managing member of and owner of a controlling

interest in OrbiMed Advisors, L.L.C. Accordingly, OrbiMed Advisors, L.L.C. and Mr. Isaly may be deemed to have voting and investment power over the shares held by OrbiMed Private Investments IV, L.P. and OrbiMed Advisors, L.L.C. Mr. Isaly disclaims beneficial ownership with respect to such shares, except to the extent of their pecuniary interest therein, if any. The address for OrbiMed Private Investments IV, L.P. is 767 3rd Avenue, 30th Floor, New York, NY 10017.

## (4)

Consists of 1,218,298 shares of common stock held by Tang Capital Partners, L.P. Excludes, in the case of shares beneficially owned before the offering and for purposes of calculating the percentage of total voting power before the offering, 365,488 shares subject to warrants that are exercisable within 60 days of August 31, 2013 by Tang Capital Partners, L.P. and, for purposes of calculating the percentage of total voting power after the offering, 252,368 shares subject to warrants that are exercisable within 60 days of August 31, 2013 by Tang Capital Partners, L.P. The warrants are not exercisable to the extent that any such exercise would increase the stockholder's ownership percentage in excess of 9.99% of our outstanding common stock, except in limited circumstances. Tang Capital Management, L.L.C., is the General Partner of Tang Capital Partners, L.P., and may be deemed to beneficially own Tang Capital Partner L.P.'s shares of common stock and shares subject to warrants that are exercisable within 60 days of Tang Capital Partners, L.P. is 47 Executive Drive, Suite 510 San Diego, CA 92121.

(5)

Includes 74,734 shares of common stock and 22,420 shares subject to warrants that are exercisable within 60 days of August 31, 2013 by QVT Fund IV L.P., 438,140 shares of common stock and 131,442 shares subject to warrants that are exercisable within 60 days of August 31, 2013 by QVT Fund V L.P., 63,457 shares subject to warrants that are exercisable within 60 days of August 31, 2013 by QVT Fund L.P. and 80,104 shares of common stock and 24,031 shares subject to warrants that are exercisable within 60 days of August 31, 2013 by Quintessence Fund L.P. The warrants are not exercisable to the extent that any such exercise would increase the stockholder's beneficial ownership percentage in excess of 9.99% of our outstanding common stock, except in limited circumstances. QVT Financial L.P. is the investment manager to QVT Fund L.P., QVT Fund IV L.P., QVT Fund V L.P. and Quintessence Fund L.P. and may be deemed to beneficially own the shares of common stock and shares subject to warrants that are exercisable within 60 days of August 31, 2013 held by QVT Fund L.P., QVT Fund IV L.P., QVT Fund V L.P. and Quintessence Fund L.P. QVT Financial G.P. L.L.C., as general partner of QVT Financial L.P., may be deemed to beneficially own the shares of common stock and shares subject to warrants that are exercisable within 60 days of August 31, 2013 beneficially owned by QVT Financial L.P. QVT Associates G.P. L.L.C., as general partner of QVT Fund L.P., QVT Fund IV L.P., QVT Fund V L.P. and Quintessence Fund L.P., also may be deemed to beneficially own the shares of common stock and shares subject to warrants that are exercisable within 60 days of August 31, 2013 owned by QVT Fund L.P., QVT Fund IV L.P., QVT Fund V L.P. and Quintessence Fund L.P. The address for QVT Fund, L.P. is c/o Walkers, 87 Mary Street, George Town, Grand Cayman KY 1-9005 Cayman Islands.

(6)

Includes 177,900 shares of common stock and 53,370 shares subject to warrants that are exercisable within 60 days of August 31, 2013 by BVF Investments LLC; 213,122 shares of common stock and 64,357 shares subject to warrants that are exercisable within 60 days of August 31, 2013 by Biotechnology Value Fund L.P.; 122,684 shares of common stock and 37,191 shares subject to warrants that are exercisable within 60 days of August 31, 2013 by Biotechnology Value Fund II L.P.; and 37,058 shares of common stock and 24,178 shares subject to warrants that are exercisable within 60 days of August 31, 2013 by Investment 10 LLC. The warrants are not exercisable to the extent that any such exercise would increase the stockholder's ownership percentage in excess of 9.99% of our outstanding common stock, except in limited circumstances. The address

for BVF Investments, L.L.C. is One Sansome Street, 30th Floor, San Francisco, CA 94104.

## (7)

Includes 345,379 shares of common stock and 103,613 shares subject to warrants that are exercisable within 60 days of August 31, 2013 by RA Capital Healthcare Fund, L.P. and 206,344 shares of common stock and 61,903 shares subject to warrants that are exercisable within 60 days of August 31, 2013 by Blackwell Partners, LLC. RA Capital Management, L.L.C. is the general partner of RA Capital Healthcare Fund, L.P. and the investment adviser of Blackwell Partners, LLC. Peter Kolchinsky is the sole manager of RA Capital Management, LLC and Mr. Kolchinsky may be deemed to have voting and investment power over the shares held by RA Capital Healthcare Fund, L.P. and Blackwell Partners, LLC. Mr. Kolchinsky disclaims beneficial ownership with respect to such shares, except to the extent of their pecuniary interest therein, if any. The address for RA Capital Healthcare Fund, L.P. is 20 Park Plaza, Suite 1200, Boston, MA 02116.

## (8)

Includes 12,378 shares subject to options exercisable within 60 days of August 31, 2013. Also includes 2,055 shares owned by Hodran Consultants Inc., of which Dr. Godbout may be deemed to share voting and investment control. The address for Martin Godbout is 4 Jardins Merici, Quebec, Quebec, G1S 4M4, Canada.

## (9)

Includes 3,582 shares subject to options exercisable within 60 days of August 31, 2013.

## (10)

Includes 79,600 shares subject to options exercisable within 60 days of August 31, 2013.

## (11)

Includes 5,782 shares subject to options exercisable within 60 days of August 31, 2013.

## (12)

Includes 3,582 shares subject to options exercisable within 60 days of August 31, 2013.

## (13)

In September 2012, Mr. Grubsztajn resigned as our President and Chief Executive Officer.

## (14)

Includes 33,066 shares subject to options exercisable within 60 days of August 31, 2013.

## (15)

Includes 670 shares subject to options exercisable within 60 days of August 31, 2013. In April 2013, Dr. Besterman resigned as our Executive Vice President Research and Development and Chief Scientific Officer.

## (16)

Includes the shares and shares subject to options exercisable within 60 days of August 31, 2013 referred to in footnotes (8), (9), (10), (11), (12) and (14), and 6,000 shares subject to options exercisable within 60 days of August 31, 2013 held by Jamie A. Donadio. It does not include shares referred to in footnotes (13) and (15), since Mr. Grubsztajn and Dr. Besterman are not current executive officers.

## DESCRIPTION OF CAPITAL STOCK

Our amended and restated certificate of incorporation authorizes us to issue up to 100,000,000 shares of common stock, \$0.001 par value, and 10,000,000 shares of preferred stock, \$0.001 par value.

As of September 30, 2013, there were:

9,960,621 shares of common stock outstanding;

800,239 shares of common stock subject to outstanding options; and

2,730,549 shares of common stock subject to outstanding warrants.

As of September 30, 2013, we had 19 holders of record of our common stock. The actual number of stockholders is greater than this number of record holders, and includes stockholders who are beneficial owners, but whose shares are held in street name by brokers and other nominees. This number of holders of record also does not include stockholders whose shares may be held in trust by other entities.

The following description of our capital stock is not complete and is subject to and qualified in its entirety by our amended and restated certificate of incorporation and amended and restated bylaws and by the provisions of applicable Delaware law.

#### **Common Stock**

As of September 30, 2013, we had 100,000,000 authorized shares of common stock, par value \$0.001 per share.

As of September 30, 2013, there were 9,960,621 shares of common stock outstanding. As of September 30, 2013, there were 3,530,788 shares of common stock subject to outstanding options and warrants to purchase shares of common stock. Each holder of common stock is entitled to one vote for each share of common stock held on all matters submitted to a vote of the stockholders, including the election of directors. Our amended and restated certificate of incorporation and bylaws do not provide for cumulative voting rights. Other than as described below, holders of our common stock have no preemptive, conversion or subscription rights, and there are no redemption or sinking fund provisions applicable to the common stock. The rights, preferences and privileges of the holders of common stock are subject to, and may be adversely affected by, the rights of the holders of shares of any series of our preferred stock that are outstanding or that we may designate and issue in the future. All of our outstanding shares of common stock are fully paid and nonassessable.

#### **Preferred Stock**

As of September 30, 2013, we had 10,000,000 authorized shares of preferred stock, par value \$0.001 per share.

As of September 30, 2013, there were no shares of preferred stock outstanding. Our Board of Directors may authorize the issuance of shares of preferred stock from time to time in one or more series, each series comprising the number of shares, designation, rights, privileges, restrictions and conditions determined by our Board of Directors. The preferred shares may have voting or conversion rights that could have the effect of restricting dividends on our shares of common stock, diluting the voting power of our shares of common stock, impairing the rights of our shares of common stock in the event of our dissolution, liquidation or winding-up or otherwise adversely affect the rights of holders of our shares of common stock. The issuance of preferred shares, while providing flexibility in connection with possible acquisitions and other corporate purposes, could, among other things, have the effect of delaying, deferring or preventing a change of control and may adversely affect the market price of our shares of common stock and may preclude stockholders from realizing a potential premium over the market value of their shares. The holders of preferred shares are entitled to receive notice of any meeting of our stockholders and to attend and vote, except as otherwise

provided in the rights and restrictions attached to the shares by the Board of Directors. As at the date hereof, there were no preferred shares issued and outstanding.

#### Warrants

As of September 30, 2013, there were warrants to purchase 2,730,549 shares of common stock outstanding, which expire between April 2016 and November 2017. Each of these warrants entitles the holder to purchase one share of common stock at prices ranging between CND\$7.46 (or US\$7.09, as converted) and CND\$8.70 (or US\$8.27, as converted), per share of common stock. Each of these warrants has a net exercise provision under which its holder may, in lieu of payment of the exercise price in cash, surrender the warrant and receive a net amount of shares based on the fair market value of our shares of common stock at the time of exercise of the warrant after deduction of the aggregate exercise price. Each of these warrants also contains provisions for the adjustment of the exercise price and the aggregate number of shares issuable upon the exercise of the warrant in the event of dividends, stock splits, reorganizations and reclassifications and consolidations. Certain of these warrants may be subject to an acceleration of their expiration dates if certain conditions are met.

#### **Pre-Emptive Rights**

Under the terms of the 2012 Securities Purchase Agreement and the 2011 Securities Purchase Agreement, certain investors, including Baker Brothers, Tavistock, OrbiMed Private Investments IV, L.P., Tang Capital Partners, L.P., QVT Fund, L.P., and RA Capital Healthcare Fund, L.P., have pre-emptive rights with respect to any proposed future issuances of our securities. In the event that the we propose to issue any class or series of our equity securities, any voting securities, or any securities convertible or exchangeable into, or entitling purchase of, any of the foregoing, we must provide written notice to each investor that purchased, together with such investor's affiliates, at least CND\$4.0 million (or US\$3.8 million, as converted) of the units sold under the 2012 Securities Purchase Agreement or CND\$3.0 million (or US\$2.9 million, as converted) of the units sold under the 2011 Securities Purchase Agreement, specifying the terms and conditions of the proposed issue. Each such investor has the right, by written notice within four business days from the date of receipt of our notice, in the case of a private placement, or within two business days from the date of the receipt of our notice in the case of a public offering, to subscribe for up to their pro rata share of offered securities, which share is calculated in proportion to the aggregate holding of securities by such investor in relation to the total number of securities issued and outstanding immediately prior to the issuance of offered securities.

The pre-emptive rights described above continue until, in the case of pre-emptive rights arising under the 2012 Securities Purchase Agreement, November 12, 2016 and, in the case of pre-emptive rights arising under the 2011 Securities Purchase Agreement, April 4, 2015. Such rights do not apply, however, to issuances of securities pursuant to:

our Stock Option Plan, the 2013 Plan or the ESPP;

any collaboration agreements entered into by us;

a public offering of our securities at a price per security at least 100% greater than the respective subscription price, which is CND\$12.44 (or US\$12.00, as converted as of October 15, 2013) in the case of pre-emptive rights arising under the 2011 Securities Purchase Agreement and CND\$14.50 (or US\$13.98, as converted as of October 15, 2013) in the case of pre-emptive rights arising under the 2012 Securities Purchase Agreement, in connection with which the securities being sold are listed on the New York Stock Exchange or the NASDAQ Stock Market, for total proceeds of at least CND\$50,000,000 (or US\$48,210,000, as converted as of October 15, 2013) and conducted by a recognized, full service investment banking firm;



in the case of pre-emptive rights arising under the 2012 Securities Purchase Agreement, the exercise of the warrants issued under such agreement; or

in the case of pre-emptive rights arising under the 2011 Securities Purchase Agreement, the exercise of warrants issued under such agreement.

Baker Brothers and Tavistock also have a right to acquire any offered securities that are subject to the pre-emptive rights but which are not otherwise purchased by an eligible investor pursuant to such pre-emptive rights. Each of the Baker Brothers and Tavistock may exercise its right to purchase such shares by stating in its notice of exercise of pre-emptive rights that it will acquire up to its pro rata share of such securities. In the event that either Baker Brothers or Tavistock does not exercise its additional rights, the other such stockholder has the right to acquire the remaining offered securities that are subject to the pre-emptive rights but that are not otherwise purchased by the other investors.

Under the terms of the 2012 Securities Purchase Agreement, for any investor that qualified for pre-emptive rights or additional rights under the 2011 Securities Purchase Agreement that also qualified for such rights under the 2012 Securities Purchase Agreement, the terms of such investor's pre-emptive rights or additional rights as applied to all shares acquired under the 2011 Securities Purchase Agreement and the 2012 Securities Purchase Agreement are governed solely by the terms of the 2012 Securities Purchase Agreement.

#### **Board Observer and Nomination Rights**

As long as either Baker Brothers or Tavistock beneficially owns at least 10% of our issued and outstanding shares of common stock, calculated on a partially diluted basis (assuming only the exercise of any convertible securities or rights to acquire shares of common stock of such stockholders), then Baker Brothers and Tavistock, as the case may be, has the right to appoint an observer to the Board of Directors. Each observer has the right to receive notice of and attend the meetings of the Board of Directors, and has the right to address the Board of Directors at any of its meetings, but does not have any right to vote at any meeting of the Board of Directors.

In addition to appointing an observer, as long as either Baker Brothers or Tavistock owns at least 10% of the issued and outstanding shares of common stock, calculated on a partially diluted basis (assuming only the exercise of any convertible securities or rights to acquire shares of common stock of such stockholders), then Baker Brothers and Tavistock, as the case may be, has the right, but not the obligation, to nominate one person to the Board of Directors. We are required to include each of Baker Brothers' and Tavistock's director nominees in our proposed slate of directors at each annual or special (if applicable) meeting and recommend that stockholders vote in favor of such nominee.

#### **Anti-Takeover Provisions**

Our amended and restated certificate of incorporation and bylaws contain provisions that might have an anti-takeover effect. These provisions, which are summarized below, may have the effect of delaying, deterring or preventing a change in control of our company. They could also impede a transaction in which our stockholders might receive a premium over the then-current market price of our common stock and our stockholders' ability to approve transactions that they consider to be in their best interests.

Our amended and restated certificate of incorporation permits our Board of Directors to issue preferred stock. We could authorize the issuance of a series of preferred stock which would grant to holders preferred rights to our assets upon liquidation, the right to receive dividend coupons before dividends would be declared to holders of shares of our existing preferred stock and our existing preferred stock and common stock. Our current stockholders have no redemption rights. In addition, as we have a large number of authorized but unissued shares, our Board of Directors could issue large blocks of voting stock to fend off unwanted tender offers or hostile takeovers without further stockholder approval.

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We are subject to Section 203 of the Delaware General Corporation Law. In general, Section 203, subject to specific exceptions, prohibits a publicly held Delaware corporation from engaging in any "business combination" with any "interested stockholder" for a period of three years following the date that the stockholder became an interested stockholder, unless:

prior to that date, the Board of Directors approved either the business combination or the transaction that resulted in the stockholder becoming an interested stockholder;

upon consummation of the transaction that resulted in the stockholder becoming an interested stockholder, the interested stockholder owned at least 85 percent of the voting stock of the corporation outstanding at the time the transaction commenced, excluding for purposes of determining the number of shares outstanding those shares owned by directors, officers and specific employee stock plans; or

on or after that date, the business combination is approved by the Board of Directors and authorized at an annual or special meeting of stockholders, and not by written consent, by the affirmative vote of the holders of at least  $66^{2}/_{3}$  percent of the outstanding voting stock that is not owned by the interested stockholder.

Section 203 defines "business combination" to include:

any merger or consolidation involving the corporation and the interested stockholder;

any sale, lease, exchange, mortgage, transfer, pledge or other disposition of 10 percent or more of the assets of the corporation involving the interested stockholder;

subject to limited exceptions, any transaction that results in the issuance or transfer by the corporation of any stock of the corporation to the interested stockholder;

any transaction involving the corporation that has the effect of increasing the proportionate share of the corporation's stock of any class or series beneficially owned by the interested stockholder; and

the receipt by the "interested stockholder" of the benefit of any loans, advances, guarantees, pledges or other financial benefits provided by or through the corporation.

In general, an "interested stockholder" is an entity or individual who, together with affiliates and associates, owns, or within three years prior to the determination of the "interested stockholder" status owned, 15 percent or more of a corporation's outstanding voting stock.

The provisions of Section 203 could encourage companies interested in acquiring us to negotiate in advance with our Board of Directors since the stockholder approval requirement would be avoided if our Board of Directors approves either the business combination or the transaction that results in the stockholder becoming an interested stockholder. These provisions also could have the effect of preventing changes in our management or could make it more difficult to accomplish transactions that stockholders may otherwise deem to be in their best interests.

#### NASDAQ Capital Market Listing

Our common stock is listed on The NASDAQ Capital Market under the symbol "MRTX."

## **Transfer Agent and Registrar**

# Edgar Filing: Schnuck Todd R - Form 4

The transfer agent and registrar for our common stock is Computershare Trust Company, N.A. The transfer agent and registrar's address is 250 Royall Street, Canton, MA, 02021.

### SHARES ELIGIBLE FOR FUTURE SALE

Prior to our registration of our common stock with the SEC in July 2013, there was no U.S. public market for our common stock, and a liquid trading market for our common stock may not develop or be sustained after this offering. Future sales of substantial amounts of our common stock in the public market, including shares issued upon exercise of outstanding options and warrants, or the anticipation of these sales, could adversely affect prevailing market prices from time to time and could impair our ability to raise equity capital in the future.

Upon completion of this offering, we will have 13,207,725 shares of common stock outstanding, based on the number of shares outstanding as of June 30, 2013 and assuming (1) no exercise of any options or warrants outstanding as of June 30, 2013 and (2) no exercise of the underwriters' option to purchase additional shares from us. All of these shares will be freely tradable, except that any shares held by our "affiliates," as that term is defined in Rule 144 under the Securities Act, or Rule 144, may only be sold in compliance with the limitations described below.

### Rule 144

Shares of our common stock that are held by securityholders that are or have been our affiliates at any time during the 90 days preceding the consummation of the Arrangement are restricted securities and are eligible for resale in compliance with Rule 144 or Rule 701 under the Securities Act, or Rule 701, subject to the requirements described below. "Restricted Securities," as defined under Rule 144, were issued and sold by us in reliance on exemptions from the registration requirements of the Securities Act. These shares may be sold in the public market only if registered or if they qualify for an exemption from registration, such as Rule 144 or Rule 701. Below is a summary of the requirements for sales of our common stock pursuant to Rule 144, as in effect on the date of this Registration Statement, after the effectiveness of this Registration Statement.

Affiliates are able to sell their shares of common stock under Rule 144, subject to the requirements of that rule. In general, under Rule 144, an affiliate is entitled to sell within any three-month period a number of shares that does not exceed one percent of the number of shares of our common stock then outstanding. Sales under Rule 144 are also subject to manner of sale provisions and notice requirements and to the availability of current public information about us. Persons who may be deemed to be our affiliates generally include individuals or entities that control, or are controlled by, or are under common control with, us and may include our directors and officers, as well as our significant stockholders.

#### Lock-Up Agreements

We, along with our executive officers and directors have agreed that for a period of 90 days after the date of this prospectus, without the prior written consent of Jefferies LLC and Leerink Swann LLC, subject to specified exceptions, we or they will not offer, sell, contract to sell, pledge or otherwise dispose of, directly or indirectly, any shares of our common stock or securities convertible into or exchangeable or exercisable for any shares of our common stock.

### **Equity Incentive Plans**

Shares of our common stock issued under the 2013 Plan and the ESPP are available for sale in the open market, subject to Rule 144 volume limitations and the lock-up agreements described above, if applicable.

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### MATERIAL U.S. FEDERAL INCOME TAX CONSEQUENCES TO NON-U.S. HOLDERS OF OUR COMMON STOCK

The following summary describes the material U.S. federal income tax consequences of the acquisition, ownership and disposition of our common stock acquired in this offering by Non-U.S. Holders (as defined below). This discussion does not address all aspects of U.S. federal income taxes that may be relevant to Non-U.S. Holders in light of their particular circumstances, does not deal with foreign, state and local tax consequences and does not address U.S. federal tax consequences other than income taxes. Special rules different from those described below may apply to certain Non-U.S. Holders that are subject to special treatment under the Code, such as financial institutions, insurance companies, tax-exempt organizations, tax-qualified retirement plans, broker-dealers and traders in securities, commodities or currencies, U.S. expatriates, "controlled foreign corporations," "passive foreign investment companies," corporations that accumulate earnings to avoid U.S. federal income tax, persons that hold our common stock as part of a "straddle," "hedge," "conversion transaction," "synthetic security" or integrated investment or other risk reduction strategy, holders deemed to sell our common stock under the constructive sale provisions of the Code, holders who hold or receive our common stock pursuant to the exercise of employee stock options or otherwise as compensation, holders who are subject to the alternative minimum tax or Medicare contribution tax, partnerships and other pass-through entities, and investors in such pass-through entities or an entity that is treated as a disregarded entity for U.S. federal income tax purposes (regardless of its place of organization or formation). Such Non-U.S. Holders are urged to consult their tax advisors to determine the U.S. federal, state, local and other tax consequences that may be relevant to them. Furthermore, the discussion below is based upon the provisions of the Code, and Treasury regulations, published administrative pronouncements, rulings and judicial decisions thereunder as of the date hereof. Such authorities may be repealed, revoked or modified, perhaps retroactively, so as to result in U.S. federal income tax consequences different from those discussed below. We have not requested a ruling from the U.S. Internal Revenue Service, or the IRS, with respect to the statements made and the conclusions reached in the following summary, and there can be no assurance that the IRS will agree with such statements and conclusions. This discussion assumes that the Non-U.S. Holder holds our common stock as a "capital asset" within the meaning of Section 1221 of the Code (generally, property held for investment).

The following discussion is for general information only and is not tax advice. Persons considering the purchase of our common stock pursuant to this offering should consult their tax advisors concerning the U.S. federal income tax consequences of acquiring, owning and disposing of our common stock in light of their particular situations as well as any consequences arising under the laws of any other taxing jurisdiction, including any state, local or foreign tax consequences and any U.S. federal non-income tax consequences.

For the purposes of this discussion, a "Non-U.S. Holder" is, for U.S. federal income tax purposes, a beneficial owner of our common stock that has not been excluded from this discussion and is not a U.S. Holder. A "U.S. Holder" means a beneficial owner of our common stock that is for U.S. federal income tax purposes (a) an individual who is a citizen or resident of the United States, (b) a corporation or other entity treated as a corporation created or organized in or under the laws of the United States, any state thereof or the District of Columbia, (c) an estate the income of which is subject to U.S. federal income taxation regardless of its source or (d) a trust if it (1) is subject to the primary supervision of a court within the United States and one or more U.S. persons have the authority to control all substantial decisions of the trust or (2) has a valid election in effect under applicable Treasury regulations to be treated as a U.S. person.

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### **Distributions on Our Common Stock**

Subject to the discussion below regarding backup withholding and foreign accounts, distributions, if any, made on our common stock to a Non-U.S. Holder of our common stock generally will constitute dividends for U.S. tax purposes to the extent made out of our current or accumulated earnings and profits (as determined under U.S. federal income tax principles) and will be subject to withholding tax at a 30% rate or such lower rate as may be specified by an applicable income tax treaty. To obtain a reduced rate of withholding under a treaty, a Non-U.S. Holder generally will be required to provide the applicable withholding agent with a properly executed IRS Form W-8BEN, or other appropriate form, certifying the Non-U.S. Holder's entitlement to benefits under that treaty. In the case of a Non-U.S. Holder that is an entity, Treasury regulations and the relevant tax treaty provide rules to determine whether, for purposes of determining the applicability of a tax treaty, dividends will be treated as paid to the entity or to those holding an interest in that entity. If a Non-U.S. Holder holds stock through a financial institution or other agent acting on the holder's behalf, the holder will be required to provide appropriate documentation to such agent. The holder's agent will then be required to provide certification to the applicable withholding agent, either directly or through other intermediaries. If you are eligible for a reduced rate of U.S. federal withholding tax under an income tax treaty, you should consult with your tax advisor to determine if you are able to obtain a refund or credit of any excess amounts withheld by timely filing an appropriate claim for a refund with the IRS.

We generally are not required to withhold tax on dividends paid to a Non-U.S. Holder that are effectively connected with the Non-U.S. Holder's conduct of a trade or business within the United States (and, if required by an applicable income tax treaty, are attributable to a permanent establishment that such holder maintains in the United States) if a properly executed IRS Form W-8ECI, stating that the dividends are so connected, is furnished to us (or, if stock is held through a financial institution or other agent, to such agent). In general, Non-U.S. Holders will be subject to U.S. federal income tax on such effectively connected dividends, on a net income basis at the regular graduated rates, unless a specific treaty exemption applies. A corporate Non-U.S. Holder receiving effectively connected dividends may also be subject to an additional "branch profits tax," which is imposed, under certain circumstances, at a rate of 30% (or such lower rate as may be specified by an applicable treaty) on the corporate Non-U.S. Holder's effectively connected earnings and profits, subject to certain adjustments.

To the extent distributions on our common stock, if any, exceed our current and accumulated earnings and profits, they will constitute a non-taxable return of capital and will first reduce your basis in our common stock, but not below zero, and then will be treated as gain and taxed in the same manner as gain realized from a sale or other disposition of common stock as described in the next section.

### Gain on Disposition of Our Common Stock

Subject to the discussion below regarding backup withholding and foreign accounts, a Non-U.S. Holder generally will not be subject to U.S. federal income tax with respect to gain realized on a sale or other taxable disposition of our common stock unless (a) the gain is effectively connected with a trade or business of such holder in the United States (and, if required by an applicable income tax treaty, is attributable to a permanent establishment that such holder maintains in the United States), (b) the Non-U.S. Holder is a nonresident alien individual and is present in the United States real property holding corporation," or a USRPHC, within the meaning of Code Section 897(c)(2) at any time within the shorter of the five-year period preceding such disposition or such holder's holding period. In general, we would be a USRPHC if interests in U.S. real estate constituted (by fair market value) at least half of our assets. We believe that we are not, and do not anticipate becoming, a USRPHC, however, there can be no assurance that we will not become a USRPHC in the future. Even if we are treated as a USRPHC, gain realized by a Non-U.S. Holder on a disposition of our common stock will not be subject to U.S. federal income tax so long as (1) the Non-U.S. Holder owned, directly, indirectly and



constructively, no more than 5% of our common stock at all times within the shorter of (i) the five-year period preceding the disposition or (ii) the holder's holding period and (2) our common stock is regularly traded on an established securities market. There can be no assurance that our common stock will continue to qualify as regularly traded on an established securities market.

If you are a Non-U.S. Holder described in (a) above, you will be required to pay tax on the net gain derived from the sale or other taxable disposition at regular graduated U.S. federal income tax rates, unless a specific treaty exemption applies, and corporate Non-U.S. Holders described in (a) above may be subject to the additional branch profits tax at a 30% rate or such lower rate as may be specified by an applicable income tax treaty. If you are an individual Non-U.S. Holder described in (b) above, you will be required to pay a flat 30% tax on the gain derived from the sale or other taxable disposition, which gain may be offset by U.S. source capital losses (even though you are not considered a resident of the United States).

#### Information Reporting Requirements and Backup Withholding

Generally, we or certain financial middlemen must report information to the IRS with respect to any dividends we pay on our common stock including the amount of any such dividends, the name and address of the recipient, and the amount, if any, of tax withheld. A similar report is sent to the holder to whom any such dividends are paid. Pursuant to tax treaties or certain other agreements, the IRS may make its reports available to tax authorities in the recipient's country of residence.

Dividends paid by us (or our paying agents) to a Non-U.S. Holder may also be subject to U.S. backup withholding. U.S. backup withholding generally will not apply to a Non-U.S. Holder who provides a properly executed IRS Form W-8BEN or otherwise establishes an exemption. The current backup withholding rate is 28%.

Under current U.S. federal income tax law, U.S. information reporting and backup withholding requirements generally will apply to the proceeds from a disposition of our common stock effected by or through a U.S. office of any broker, U.S. or foreign, except that information reporting and such requirements may be avoided if the holder provides a properly executed IRS Form W-8BEN or otherwise meets documentary evidence requirements for establishing Non-U.S. Holder status or otherwise establishes an exemption. Generally, U.S. information reporting and backup withholding requirements will not apply to a payment of disposition proceeds to a Non-U.S. Holder where the transaction is effected outside the United States through a non-U.S. office of a non-U.S. broker. For information reporting purposes, certain brokers with substantial U.S. ownership or operations will generally be treated in a manner similar to U.S. brokers.

If backup withholding is applied to you, you should consult with your tax advisor to determine if you are able to obtain a tax benefit or credit with respect to such backup withholding.

### **Foreign Accounts**

A U.S. federal withholding tax of 30% may apply to dividends paid after June 30, 2014 and the gross proceeds from a disposition of our common stock paid after December 31, 2016 to a foreign financial institution (as specifically defined for this purpose), including when the foreign financial institution holds our common stock on behalf of a non-U.S. Holder, unless such institution enters into an agreement with the U.S. government to withhold on certain payments and to collect and provide to the U.S. tax authorities substantial information regarding U.S. account holders of such institution (which may include certain equity and debt holders of such institution, as well as certain account holders that are foreign entities with U.S. owners). This U.S. federal withholding tax of 30% will also apply to dividends paid after June 30, 2014 and the gross proceeds from a disposition of our common stock paid after December 31, 2016 to a non-financial foreign entity unless such entity provides the withholding agent with either a certification that it does not have any substantial direct or indirect U.S. owners or provides information regarding direct and indirect U.S. owners of the entity. Under certain circumstances, a Non-U.S. Holder might be eligible for refunds or credits of such taxes. An intergovernmental agreement between the United States and an



applicable foreign country may modify the requirements described in this paragraph. Holders are encouraged to consult with their tax advisors regarding the possible implications of the legislation on their investment in our common stock.

THE PRECEDING DISCUSSION OF U.S. FEDERAL INCOME TAX CONSIDERATIONS IS FOR GENERAL INFORMATION ONLY. IT IS NOT TAX ADVICE. EACH PROSPECTIVE INVESTOR SHOULD CONSULT ITS TAX ADVISOR REGARDING THE TAX CONSEQUENCES OF PURCHASING, HOLDING AND DISPOSING OF OUR COMMON STOCK, INCLUDING THE CONSEQUENCES OF ANY PROPOSED CHANGE IN APPLICABLE LAW.

### UNDERWRITING

Subject to the terms and conditions set forth in the underwriting agreement, dated October 23, 2013, among us and Jefferies LLC and Leerink Swann LLC, as the representatives of the underwriters named below and the joint book-running managers of this offering, we have agreed to sell to the underwriters, and each of the underwriters has agreed, severally and not jointly, to purchase from us, the respective number of shares of common stock shown opposite its name below:

Underwriter	Number of Shares
Jefferies LLC	1,787,500
Leerink Swann LLC	975,000
Piper Jaffray & Co.	487,500
Total	3,250,000

The underwriting agreement provides that the obligations of the several underwriters are subject to certain conditions precedent such as the receipt by the underwriters of officers' certificates and legal opinions and approval of certain legal matters by their counsel. The underwriting agreement provides that the underwriters will purchase all of the shares of common stock if any of them are purchased. If an underwriter defaults, the underwriting agreement provides that the purchase commitments of the nondefaulting underwriters may be increased or the underwriting agreement may be terminated. We have agreed to indemnify the underwriters and certain of their controlling persons against certain liabilities, including liabilities under the Securities Act, and to contribute to payments that the underwriters may be required to make in respect of those liabilities.

The underwriters have advised us that, following the completion of this offering, they currently intend to make a market in the common stock as permitted by applicable laws and regulations. However, the underwriters are not obligated to do so, and the underwriters may discontinue any market-making activities at any time without notice in their sole discretion. Accordingly, no assurance can be given as to the liquidity of the trading market for the common stock, that you will be able to sell any of the common stock held by you at a particular time or that the prices that you receive when you sell will be favorable.

The underwriters are offering the shares of common stock subject to their acceptance of the shares of common stock from us and subject to prior sale. The underwriters reserve the right to withdraw, cancel or modify offers to the public and to reject orders in whole or in part.

### **Commission and Expenses**

The underwriters have advised us that they propose to offer the shares of common stock to the public at the offering price set forth on the cover page of this prospectus and to certain dealers, which may include the underwriters, at that price less a concession not in excess of \$0.63 per share of common stock. After the offering, the offering price, concession and reallowance to dealers may be reduced by the representatives. No such reduction will change the amount of proceeds to be received by us as set forth on the cover page of this prospectus.

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The following table shows the public offering price, the underwriting discounts and commissions that we are to pay the underwriters and the proceeds, before expenses, to us in connection with this offering. Such amounts are shown assuming both no exercise and full exercise of the underwriters' option to purchase additional shares.

	W	Per S ithout	Share		Total				
	Option to Purchase		Option With		Without		With		
			to Option to			Option to	Option to		
			nase Purchase		]	Purchase	Purchase		
	Add	ditional Additional		Additional		A	Additional		
	Shares		Shares Shares		Shares		Shares		
Public offering price	\$	17.50	\$	17.50	\$	56,875,000	\$	65,406,250	
Underwriting discounts and commissions paid by us	\$	1.05	\$	1.05	\$	3,412,500	\$	3,924,375	
Proceeds to us, before expenses	\$	16.45	\$	16.45	\$	53,462,500	\$	61,481,875	

We estimate expenses payable by us in connection with this offering, other than the underwriting discounts and commissions referred to above, will be approximately \$500,000. We have also agreed to reimburse the underwriters for certain other expenses in an amount not to exceed \$55,000 as set forth in the underwriting agreement.

#### Listing

Our common stock is listed on The NASDAQ Capital Market under the trading symbol "MRTX."

#### **Option to Purchase Additional Shares**

We have granted to the underwriters an option, exercisable for 30 days from the date of this prospectus, to purchase, from time to time, in whole or in part, up to an aggregate of 487,500 shares from us at the public offering price set forth on the cover page of this prospectus, less underwriting discounts and commissions. If the underwriters exercise this option, each underwriter will be obligated, subject to specified conditions, to purchase a number of additional shares proportionate to that underwriter's initial purchase commitment as indicated in the table above. This option may be exercised only if the underwriters sell more shares than the total number set forth on the cover page of this prospectus.

#### No Sales of Similar Securities

We, our executive officers and directors have agreed, subject to specified exceptions, not to directly or indirectly:

sell, offer, contract or grant any option to sell (including any short sale), pledge, transfer, establish an open "put equivalent position" within the meaning of Rule 16a-l(h) under the Exchange Act, or

otherwise dispose of any shares of common stock, options or warrants to acquire shares of common stock, or securities exchangeable or exercisable for or convertible into shares of common stock currently or hereafter owned either of record or beneficially, or

publicly announce an intention to do any of the foregoing for a period of 90 days after the date of this prospectus without the prior written consent of Jefferies LLC and Leerink Swann LLC.

This restriction terminates after the close of trading of the common stock on and including the 90th day after the date of this prospectus.

Jefferies LLC and Leerink Swann LLC may, in their sole discretion and at any time or from time to time before the termination of the 90-day period release all or any portion of the securities subject to lock-up agreements. There are no existing agreements between the underwriters and

# Explanation of Responses:

any of our stockholders who

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will execute a lock-up agreement, providing consent to the sale of shares prior to the expiration of the lock-up period.

#### Stabilization

The underwriters have advised us that, pursuant to Regulation M under the Exchange Act, certain persons participating in the offering may engage in short sale transactions, stabilizing transactions, syndicate covering transactions or the imposition of penalty bids in connection with this offering. These activities may have the effect of stabilizing or maintaining the market price of the common stock at a level above that which might otherwise prevail in the open market. Establishing short sales positions may involve either "covered" short sales or "naked" short sales.

"Covered" short sales are sales made in an amount not greater than the underwriters' option to purchase additional shares of our common stock in this offering. The underwriters may close out any covered short position by either exercising their option to purchase additional shares of our common stock or purchasing shares of our common stock in the open market. In determining the source of shares to close out the covered short position, the underwriters will consider, among other things, the price of shares available for purchase in the open market as compared to the price at which they may purchase shares through the option to purchase additional shares.

"Naked" short sales are sales in excess of the option to purchase additional shares of our common stock. The underwriters must close out any naked short position by purchasing shares in the open market. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of the shares of our common stock in the open market after pricing that could adversely affect investors who purchase in this offering.

A stabilizing bid is a bid for the purchase of shares of common stock on behalf of the underwriters for the purpose of fixing or maintaining the price of the common stock. A syndicate covering transaction is the bid for or the purchase of shares of common stock on behalf of the underwriters to reduce a short position incurred by the underwriters in connection with the offering. Similar to other purchase transactions, the underwriters' purchases to cover the syndicate short sales may have the effect of raising or maintaining the market price of our common stock or preventing or retarding a decline in the market price of our common stock. As a result, the price of our common stock may be higher than the price that might otherwise exist in the open market. A penalty bid is an arrangement permitting the underwriters to reclaim the selling concession otherwise accruing to a syndicate member in connection with the offering if the common stock originally sold by such syndicate member.

Neither we, nor any of the underwriters make any representation or prediction as to the direction or magnitude of any effect that the transactions described above may have on the price of our common stock. The underwriters are not obligated to engage in these activities and, if commenced, any of the activities may be discontinued at any time.

The underwriters may also engage in passive market making transactions in our common stock on The NASDAQ Capital Market in accordance with Rule 103 of Regulation M during a period before the commencement of offers or sales of shares of our common stock in this offering and extending through the completion of distribution. A passive market maker must display its bid at a price not in excess of the highest independent bid of that security. However, if all independent bids are lowered below the passive market maker's bid, that bid must then be lowered when specified purchase limits are exceeded.

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### **Electronic Distribution**

A prospectus in electronic format may be made available by e-mail or on the web sites or through online services maintained by one or more of the underwriters or their affiliates. In those cases, prospective investors may view offering terms online and may be allowed to place orders online. The underwriters may agree with us to allocate a specific number of shares of common stock for sale to online brokerage account holders. Any such allocation for online distributions will be made by the underwriters on the same basis as other allocations. Other than the prospectus in electronic format, the information on the underwriters' web sites and any information contained in any other web site maintained by any of the underwriters is not part of this prospectus, has not been approved and/or endorsed by us or the underwriters and should not be relied upon by investors.

#### **Other Activities and Relationships**

The underwriters and certain of their respective affiliates are full service financial institutions engaged in various activities, which may include securities trading, commercial and investment banking, financial advisory, investment management, investment research, principal investment, hedging, financing and brokerage activities. The underwriters and certain of their respective affiliates have, from time to time, performed, and may in the future perform, various commercial and investment banking and financial advisory services for us and our affiliates, for which they received or will receive customary fees and expenses. In particular, Jefferies LLC has acted as a broker in connection with certain of our private financings.

In the ordinary course of their various business activities, the underwriters and certain of their respective affiliates may make or hold a broad array of investments and actively trade debt and equity securities (or related derivative securities) and financial instruments (including bank loans) for their own account and for the accounts of their customers, and such investment and securities activities may involve securities and/or instruments issued by us and our affiliates. If the underwriters or their respective affiliates have a lending relationship with us, they routinely hedge their credit exposure to us consistent with their customary risk management policies. The underwriters and their respective affiliates may hedge such exposure by entering into transactions which consist of either the purchase of credit default swaps or the creation of short positions in our securities or the securities of the common stock offered hereby. Any such short positions could adversely affect future trading prices of the common stock offered hereby. The underwriters and certain of their respective affiliates may also communicate independent investment recommendations, market color or trading ideas and/or publish or express independent research views in respect of such securities or instruments and may at any time hold, or recommend to clients that they acquire, long and/or short positions in such securities and instruments.

### **Disclaimers About Non-U.S. Jurisdictions**

#### European Economic Area

In relation to each Member State of the European Economic Area which has implemented the Prospectus Directive (each, a "Relevant Member State") an offer to the public of any shares which are the subject of the offering contemplated by this prospectus may not be made in that Relevant Member State except that an offer to the public in that Relevant Member State of any shares may be made at any time under the following exemptions under the Prospectus Directive, if they have been implemented in that Relevant Member State:

#### (a)

to legal entities which are authorized or regulated to operate in the financial markets or, if not so authorized or regulated, whose corporate purpose is solely to invest in securities;

#### (b)

to any legal entity which has two or more of (1) an average of at least 250 employees during the last financial year; (2) a total balance sheet of more than  $\notin$ 43,000,000 and (3) an annual net turnover of more than  $\notin$ 50,000,000, as shown in its last annual or consolidated accounts;

(c)

to fewer than 100 natural or legal persons (other than qualified investors as defined in the Prospectus Directive) subject to obtaining the prior consent of the representatives for any such offer; or

(d)

in any other circumstances falling within Article 3(2) of the Prospectus Directive, provided that no such offer of the shares shall result in a requirement for the publication by us or any underwriter of a prospectus pursuant to Article 3 of the Prospectus Directive.

Each person in a Relevant Member State who receives any communication in respect of, or who acquires any shares under, the offers contemplated in this prospectus will be deemed to have represented, warranted and agreed to and with each underwriter and us that:

(a)

it is a qualified investor within the meaning of the law in that Relevant Member State implementing Article 2(1)(e) of the Prospectus Directive; and

#### (b)

in the case of any shares acquired by it as a financial intermediary, as that term is used in Article 3(2) of the Prospectus Directive, (i) the shares acquired by it in the offer have not been acquired on behalf of, nor have they been acquired with a view to their offer or resale to, persons in any Relevant Member State, other than qualified investors, as that term is defined in the Prospectus Directive, or in circumstances in which the prior consent of the representatives has been given to the offer or resale; or (ii) where shares have been acquired by it on behalf of persons in any Relevant Member State other than qualified investors, the offer of those shares to it is not treated under the Prospectus Directive as having been made to such persons.

For the purposes of this provision, the expression an "offer to the public" in relation to any shares in any Relevant Member State means the communication in any form and by any means of sufficient information on the terms of the offer and any shares to be offered so as to enable an investor to decide to purchase any shares, as the same may be varied in that Member State by any measure implementing the Prospectus Directive in that Member State and the expression "Prospectus Directive" means Directive 2003/71/EC and includes any relevant implementing measure in each Relevant Member State.

### United Kingdom

Each underwriter has represented, warranted and agreed that:

(a)

it has only communicated or caused to be communicated and will only communicate or cause to be communicated any invitation or inducement to engage in investment activity (within the meaning of Section 21 of the Financial Services and Markets Act 2000, or the FSMA) to persons who are investment professionals falling within Article 19(5) of the FSMA (Financial Promotion) Order 2005 or in circumstances in which Section 21(1) of the FSMA does not apply to us; and

(b)

it has complied with and will comply with all applicable provisions of the FSMA with respect to anything done by it in relation to the shares in, from or otherwise involving the United Kingdom.

### LEGAL MATTERS

The validity of the shares of common stock being offered by this prospectus will be passed upon for us by Cooley LLP, San Diego, California. The underwriters are being represented by Latham & Watkins LLP, San Diego, California.

### EXPERTS

Ernst & Young LLP, independent registered public accounting firm, has audited our consolidated financial statements included elsewhere in this prospectus, as set forth in their report included elsewhere in this prospectus. Our consolidated financial statements are included in this prospectus in reliance on Ernst & Young LLP's report, given on their authority as experts in accounting and auditing.

### WHERE YOU CAN FIND ADDITIONAL INFORMATION

We have filed with the SEC a registration statement on Form S-1 under the Securities Act, with respect to the shares of common stock being offered by this prospectus. This prospectus does not contain all of the information in the registration statement and its exhibits. For further information with respect to us and the common stock offered by this prospectus, we refer you to the registration statement and its exhibits. Statements contained in this prospectus as to the contents of any contract or any other document referred to are not necessarily complete, and in each instance, we refer you to the copy of the contract or other document filed as an exhibit to the registration statement. Each of these statements is qualified in all respects by this reference.

You can read our SEC filings, including the registration statement, over the Internet at the SEC's website at www.sec.gov. You may also read and copy any document we file with the SEC at its public reference facilities at 100 F Street NE, Washington, D.C. 20549. You may also obtain copies of these documents at prescribed rates by writing to the Public Reference Section of the SEC at 100 F Street NE, Washington, D.C. 20549. Please call the SEC at 1-800-SEC-0330 for further information on the operation of the public reference facilities. You may also request a copy of these filings, at no cost, by writing us at 9363 Towne Centre Drive, Suite 200, San Diego, California 92121 or telephoning us at (858) 332-3410.

We are subject to the information and periodic reporting requirements of the Exchange Act, and we will file periodic reports, proxy statements and other information are available for inspection and copying at the public reference room and website of the SEC referred to above. We maintain a website at http://www.mirati.com. You may access our annual reports on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K and amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Exchange Act with the SEC free of charge at our website as soon as reasonably practicable after such material is electronically filed with, or furnished to, the SEC. The information contained in, or that can be accessed through, our website is not part of this prospectus.

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Index to Consolidated Financial Statements of Mirati Therapeutics, Inc.

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### REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

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

We have audited the accompanying consolidated balance sheets of Mirati Therapeutics, Inc. as of December 31, 2012 and 2011, and the related consolidated statements of operations and comprehensive loss, changes in stockholders' equity, and cash flows for each of the two years in the period ended December 31, 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 consolidated financial statements are free of material misstatement. We were not engaged to perform an audit of the Company's internal control over financial reporting. Our audits included consideration of internal control over financial reporting as a basis for designing audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion. An audit also includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements, assessing the accounting principles used and significant estimates made by management, and evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the financial statements referred to above present fairly, in all material respects, the consolidated financial position of Mirati Therapeutics, Inc. at December 31, 2012 and 2011, and the consolidated results of its operations and its cash flows for each of the two years in the period ended December 31, 2012, in conformity with U.S. generally accepted accounting principles.

/s/ Ernst & Young LLP(1)

Montreal, Canada May 8, 2013, except for Note 21, as to which the date is June 28, 2013

(1)

CPA auditor, CA, public accountancy permit no. A120254

### Mirati Therapeutics, Inc. CONSOLIDATED BALANCE SHEETS (in thousands)

		<b>DECEMBER 31,</b>			
		2012		2011	
ASSETS					
Current assets					
Cash and cash equivalents	\$	18,403	\$	9,882	
Marketable securities and term deposits		18,580		18,563	
Restricted cash equivalents and marketable securities		302		295	
Interest and other receivables		507		172	
Other current assets		1,537		1,548	
Total current assets		39,329		30,460	
Security deposits		67		54	
Restricted cash equivalents and marketable securities		72		349	
Property and equipment, net		333		219	
Total assets	\$	39,801	\$	31,082	
LIABILITIES AND STOCKHOLDERS' EQUITY					
Current liabilities					
Accounts payable and accrued liabilities		5,272		3,749	
Current portion of other liability		68			
Total current liabilities		5,340		3,749	
Other liability		45		28	
Total liabilities		5,385		3,777	
Commitments and contingencies					
Stockholders' equity					
Preferred stock, \$0.001 par value, 10,000,000 shares authorized; none issued and outstanding at					
December 31, 2012 and 2011, respectively					
Common stock, \$0.001 par value; 100,000,000 authorized; 9,957,739 and 6,358,267 issued and					
outstanding at December 31, 2012 and 2011, respectively		10		6	
Warrants		11,153		6,247	
Additional paid-in capital		154,224		132,312	
Accumulated other comprehensive income		9,520		8,945	
Accumulated deficit		(140,491)		(120,205)	
Total stockholders' equity		34,416		27,305	
ז טנמו אוטנגווטוענדא בעשונא		54,410		21,505	
Total liabilities and stockholders' equity	\$	39,801	\$	31,082	
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Subsequent events (*Note 21*) See accompanying notes

### Mirati Therapeutics, Inc. CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS (in thousands except for share and per share amounts)

	YEARS ENDED DECEMBER 31			R 31,
Revenue		2012		2011
Research collaborations and contract revenues	\$		\$	811
License and up-front fees	φ		φ	2,333
License and up-nont lees				2,355
Total revenue				3,144
Expenses				
Research and development, net		15,081		8,891
General and administrative		5,394		4,340
		~		12.021
Total operating expenses		20,475		13,231
Loss from operations		(20,475)		(10,087)
Other income, net		228		309
Loss before income taxes		(20,247)		(9,778)
Income tax expense		39		() -/
Net loss and comprehensive loss for the year	\$	(20,286)	\$	(9,778)
Basic and diluted net loss per share	\$	(3.00)	\$	(1.98)
Weighted average number of shares used in computing net loss per share, basic and diluted		6,762,985		4,944,184

See accompanying notes

### Mirati Therapeutics, Inc. CONSOLIDATED STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY (in thousands)

						A	ccu	imulated				
	Common	Stock	Co	ommon	Ad	ditional	(	other			r.	Fotal
			5	Stock	p	aid-inco	mp	rehensi	æc	umulate <b>st</b>	ocł	kholders
	Shares A	Amou	ıWa	arrants	c	apital	in	come	(	deficit	e	quity
Balance at January 1,						•						1 0
2011	808,372	\$ 1	\$		\$	104,010	\$	10,498	\$	(110,427)	\$	4,082
Net loss for the year										(9,778)		(9,778)
Stock-based compensation												
expense						940						940
Costs of reorganization						(33)						(33)
Issuance of common stock,												
net of costs	5,549,895	5				27,395						27,400
Issuance of warrants, net of												
costs				6,247								6,247
Foreign currency												
translation								(1,553)				(1,553)
Balance at December 31,												
2011	6,358,267	6		6,247		132,312		8,945		(120,205)		27,305
				, í		,		,				, í
Net loss for the year										(20,286)		(20,286)
Stock-based compensation										( - ) )		
expense						2,009						2,009
Costs of reorganization						(15)						(15)
Issuance of common stock.												
net of costs	3,593,819	4				19,882						19,886
Issuance of warrants, net of						- )						- ,
costs				4,942								4,942
Exercise of warrants	5,653			(36)		36						,
Foreign currency												
translation								575				575
Balance at December 31,												
2012	9,957,739	\$ 10	\$	11,153	\$	154.224	\$	9,520	\$	(140.491)	\$	34,416
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See accompanying notes

#### Mirati Therapeutics, Inc. CONSOLIDATED STATEMENTS OF CASH FLOWS (in thousands)

**YEARS ENDED DECEMBER 31.** 2012 2011 **Operating activities** Net loss for the year \$ (20,286) \$ (9,778)Non-cash adjustments reconciling net loss to operating cash flows 197 Depreciation of property and equipment 123 Write-off of property and equipment 77 Gain on disposal of property and equipment (30) Reversal of provision for lease resiliation (52) 940 Stock-based compensation expense 2,009 License and up-front fees (2,333)Lease incentive 85 28 Changes in operating assets and liabilities Interest and other receivables (331)150 Other current assets 45 (553)Accounts payable and accrued liabilities 1.434 (353)Change in provision for lease abandonment costs (346)Unbilled revenue 409 Cash flows used for operating activities (16,921) (11,644) **Investing activities** Purchase of property and equipment (230)(110)Purchases of marketable securities and term deposits (29, 431)(44, 670)Security deposit (12)61 Restricted cash equivalents and marketable securities 283 604 Disposal and maturities of marketable securities and term deposits 29,716 24,827 Proceeds from disposal of property and equipment 77 Cash flows provided by/(used for) investing activities 326 (19,211) **Financing activities** Issuance of common stock 20,966 29,032 Common stock issuance costs (1,080)(1,632)Issuance of warrants 6,619 5,180 Warrant issuance costs (238)(372)Costs of reorganization (15)(33)Cash flows provided by financing activities 24,813 33,614 8,218 Increase in cash and cash equivalents 2,759 Effect of exchange rate changes on cash and cash equivalents 303 (278)Cash and cash equivalents, beginning of year 9,882 7,401 \$ 18.403 \$ 9.882 Cash and cash equivalents, end of year Income taxes paid \$ 34 \$ Interest paid 6 1

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See accompanying notes

### Mirati Therapeutics, Inc.

#### Notes to Consolidated Financial Statements

December 31, 2012

#### 1. DESCRIPTION OF BUSINESS

Mirati Therapeutics, Inc. ("Mirati" or the "Company") is a biopharmaceutical company and its primary business purpose is to develop and commercialize novel therapeutics for cancer and infectious disease.

MethylGene US Inc., a wholly-owned subsidiary was incorporated in Princeton, New Jersey, USA on December 20, 2011, started business activity in 2012. The Company also has a wholly-owned subsidiary in Canada, MethylGene, Inc., ("MethylGene"). MethylGene's common stock has been listed on the Toronto Stock Exchange since June 29, 2004 under the ticker symbol "MYG". The Company is a holding company with minimal assets other than the stock of MethylGene and primarily conducts its operations through MethylGene and MethylGene US Inc. Refer to Note 2 under the heading Basis of presentation for further discussion of the Company's corporate structure.

### 2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

#### **Basis of presentation**

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

Mirati was incorporated under the laws of the State of Delaware on April 29, 2013. The Company was created to enter into an arrangement agreement described below.

On May 8, 2013, the Company's Board of Directors approved and the Company entered into an arrangement agreement with MethylGene. Subject to the terms and conditions of the arrangement agreement, which was consummated on June 28, 2013, the shareholders of MethylGene received one share of the Company's common stock in exchange for every 50 common shares of MethylGene, which had the effect of a 50 for 1 reverse split of the common shares pursuant to a court-approved plan of arrangement under Section 192 of the Canada Business Corporations Act. Such transaction is referred to herein as the Arrangement. In addition, all outstanding options and warrants to purchase common shares of MethylGene became exercisable on a 50-for-1 basis for shares of our common stock, and a proportionate adjustment was made to the exercise price or conversion price, as applicable. Upon completion of the Arrangement, MethylGene became the Company's wholly-owned subsidiary. The shares of the Company's common stock issued at the closing of the Arrangement were issued in reliance upon the exemption from registration under Section 3(A)(10) of the Securities Act of 1933, as amended. These financial statements reflect the completion of the Arrangement, which was consummated on June 28, 2013 (refer to note 21).

### Foreign currency translation

Foreign currency transactions are initially recorded by the Company using the exchange rates prevailing at the date of the transaction. At the balance sheet date, monetary assets and liabilities denominated in foreign currencies are translated at the period-end rates of exchange. Non-monetary assets and liabilities are translated at the historical exchange rates. Exchange gains and losses arising from the translation of foreign currency items are included in other income in the consolidated statements of operations and comprehensive loss. The Company recognized net foreign exchange losses of \$12 thousand and net foreign exchange gains of \$44 thousand in other income in the consolidated statement of operations and comprehensive loss for the years ended December 31, 2012 and 2011, respectively.

### Mirati Therapeutics, Inc.

#### Notes to Consolidated Financial Statements (Continued)

December 31, 2012

### 2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES (Continued)

#### **Reporting currency**

The Company's functional currency is the Canadian dollar and its reporting currency is the U.S. dollar. For presentation purposes, assets and liabilities are translated to U.S. dollars at exchange rates at the reporting date. Income and expenses are translated to U.S. dollars at the average exchange rate for the period in which the transactions occur. Equity transactions are translated at the spot exchange rates on the date the transactions occur. Exchange rate differences are recognized in a separate component of stockholders' equity titled accumulated other comprehensive income.

#### Cash and cash equivalents

Cash is comprised of cash on hand and cash equivalents. Cash equivalents are marketable securities comprised of bankers' acceptances and other short-term investment vehicles that are highly liquid and are readily convertible to known amounts of cash, which are subject to an insignificant risk of change in value, and have a maturity of less than 90 days from the date of purchase.

#### Marketable securities and term deposits

Marketable securities consist of bankers' acceptances and other investment vehicles that are highly liquid and are readily convertible to known amounts of cash, which are subject to an insignificant risk of change in value, and have an original maturity of greater than 90 days.

#### **Property and equipment**

Property and equipment is stated at historical cost less accumulated depreciation and/or accumulated impairment losses, if any. Historical cost includes expenditures that are directly attributable to the acquisition of the items. Subsequent costs are included in the asset's carrying amount or recognized as a separate asset, as appropriate, only when it is probable that future economic benefits associated with the item will flow to the Company and the cost of the item can be measured reliably. All other repairs and maintenance are charged to net loss during the financial period in which they are incurred.

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

Computer equipment	3 years
Office and other equipment	6 years
Laboratory equipment	6 years
Leasehold improvements	Over the life of the lease

On disposal of property and equipment, the cost and related accumulated depreciation and impairments are removed from the financial statements and the net amount, less any proceeds, is included in net loss.

### Mirati Therapeutics, Inc.

Notes to Consolidated Financial Statements (Continued)

December 31, 2012

### 2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES (Continued)

#### Impairment of property and equipment

The Company assesses its property and equipment for impairment whenever events or changes in circumstances (a "triggering event") indicate that the carrying value of a group of long-lived assets may not be recoverable. If such circumstances are determined to exist, an estimate of undiscounted future cash flows produced by the long-lived asset, including its eventual residual value, is compared to the carrying value to determine whether impairment exists. In the event that such cash flows are not expected to be sufficient to recover the carrying amount of the assets, the assets are written-down to their estimated fair values. Fair value is estimated through discounted cash flow models to project cash flows from the asset. The Company did not recognize an impairment charge related to its property and equipment during 2012 and 2011.

#### Stock-based compensation plan

The Company has a stock option compensation plan in which the fair value of stock options granted is determined at the date of the grant using the Black-Scholes option-pricing model and is expensed over the vesting period of the options. Awards with graded vesting are considered multiple awards for fair value measurement and stock-based compensation calculation. In determining the expense, the Company deducts the number of options that are expected to be forfeited at the time of a grant and revises this estimate, if necessary, in subsequent years if actual forfeitures differ from those estimated. Any amounts paid by employees on exercise of the stock options and subsequent purchase of stock are credited to common stock.

#### Common stock issue costs

Common stock issue costs incurred by the Company are recorded as a reduction of common stock.

#### **Revenue recognition**

The Company recognizes revenue from research collaboration agreements and licensing arrangements.

Revenue from research collaboration agreements recognized as separate units of accounting are recognized as the contracted services are performed, in accordance with the terms of the specific agreements and when collection is reasonably assured.

Revenue recognized but not invoiced to partners is recorded as unbilled revenue. Combined elements, including up-front payments for the use of technology where further services are to be provided or fees received on the signing of research agreements, are recognized over the period of performance of the related activities. As such, up-front licensing revenue is deferred and recognized over the term during which the Company maintains substantive contractual obligations and amounts received in advance of recognition of revenue are reported as deferred revenue.

In the event that the period of the substantive obligations changes, the appropriate adjustment will be made to the amortization of deferred revenue.

Research collaboration agreements and licensing arrangements may include multiple elements. Revenue arrangements with multiple elements are reviewed in order to determine whether the multiple elements can be divided into separate units of accounting. If separable, the consideration received is allocated among the

### Mirati Therapeutics, Inc.

#### Notes to Consolidated Financial Statements (Continued)

#### December 31, 2012

### 2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES (Continued)

separate units of accounting based on their relative fair values, and the applicable revenue recognition criteria are applied to each of the separate units. Otherwise, the applicable revenue recognition criteria are applied to combined elements as a single unit of accounting.

The Company applies a hierarchy to determine the selling price to be used for allocating revenue to deliverables as follows: (i) vendor-specific objective evidence ("VSOE") of fair value, (ii) third-party evidence of selling price ("TPE"), and (iii) best estimate of the selling price ("ESP"). Where VSOE and TPE are not available, the Company's process for determining ESP includes multiple factors that may vary depending upon the unique facts and circumstances related to each deliverable.

The Company executes collaborative agreements which may contain milestone payments. Revenue from milestones, if they are considered substantive, are recognized upon successful accomplishment of the milestones. Determining whether a milestone is substantive involves judgment, including an assessment of its involvement in achieving the milestones and whether the amount of the payment is commensurate to its performance. If not considered substantive, milestones are initially deferred and recognized over the remaining performance obligation.

#### Investment tax credits

The Company's accounts include claims for investment tax credits ("ITCs") relating to scientific research and experimental development activities of the Company. The qualification and recording of these activities for investment tax credit purposes are established by the Canadian federal and Provincial Tax Acts and are subject to audit by the taxation authorities. Refundable ITCs are reflected as reductions of expenses or reductions of the cost of the assets to which they relate when there is reasonable assurance that the assistance will be received and all conditions have been complied with. The non-refundable ITCs are carried forward to a time and will be recognized when it is more likely than not that the Company will become subject to Canadian federal taxes, at which time, said ITCs are applied as a reduction of tax expense.

### **Research and development expenses**

Research and development expenditures are charged to net loss in the period in which they are incurred and comprise of the following types of costs incurred in performing research and development activities and those incurred in connection with research and development revenue: salaries and benefits, allocated overhead and occupancy costs, clinical trial and related clinical manufacturing costs, contract services, and other outside costs.

#### **Income taxes**

Income taxes have been accounted for using the asset and liability method. Under the asset and liability method, deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the financial carrying amounts of existing assets and liabilities and their respective tax bases and operating loss and tax credit carryforwards. Deferred tax assets and liabilities are measured using enacted tax rates applicable to taxable income in the years in which those temporary differences are expected to be recovered or settled. The effect on deferred tax assets and liabilities of a change in tax rates is recognized in income in the period that includes the enactment date. A valuation allowance against deferred tax assets is recorded if, based upon the weight of all available evidence, it is more likely than not that some or all of the deferred tax assets will not be realized. For uncertain tax positions that meet "a

### Mirati Therapeutics, Inc.

#### Notes to Consolidated Financial Statements (Continued)

#### December 31, 2012

### 2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES (Continued)

more likely than not" threshold, the Company recognizes the benefit of uncertain tax positions in the consolidated financial statements.

#### Sales tax

Revenue, expenses and assets are recognized net of the amount of sales tax, except where the sales tax incurred on a purchase of assets or services that is not recoverable from the taxation authority, in which case the sales tax is recognized as part of the cost of acquisition of the asset or as part of the expense item as applicable. The net amount of sales tax recoverable from, or payable to the taxation authority is included as part of other current assets or accounts payable in the Consolidated Balance Sheet.

#### Net loss per share

Loss per share is calculated using the weighted average number of shares of common stock outstanding during the period. Diluted loss per share is calculated giving effect to the exercise of all dilutive factors, and assumes that any proceeds that could be obtained upon the exercise of options would be used to purchase shares of common stock at the average market price during the year. Common stock equivalents from stock options and warrants are excluded from the calculation of net loss per share for all periods presented because the effect is anti-dilutive.

#### **Financial instruments**

#### Fair value

Cash equivalents, marketable securities and restricted cash equivalents and marketable securities are held for trading as they are managed and their performance is evaluated on a fair value basis, in accordance with a documented risk management or investment strategy, and information about the investments is provided internally on that basis to key management personnel. Unrealized gains/losses are included in other income in the statement of operations and comprehensive loss. Transaction costs are expensed. The amortization of acquisition premiums and discounts is recorded as a deduction from or addition to interest earned on those financial assets, respectively.

#### Other financial assets

Other financial assets are initially recorded at fair value and are subsequently measured at amortized cost using the effective interest rate method less impairment. The Company's other financial assets consist of interest receivable, other receivables and security deposits. The carrying amount of these financial assets is a reasonable approximation of their fair value due to the short-term nature of these financial assets.

#### Other financial liabilities

All financial liabilities are recognized initially at fair value and subsequently measured at amortized cost. The Company's financial liabilities include accounts payable and accrued liabilities. The carrying value of the accounts payable and accrued liabilities approximates their fair value due to the short-term nature of these financial liabilities.

### **Interest Income**

Interest income earned on cash equivalents and marketable securities balances is recognized on an accrual basis as earned and reported in other income in the statement of operations and comprehensive loss. The Company recognized net interest income of \$0.2 million and \$0.3 million for the years ended December 31, 2012 and 2011, respectively.

### Mirati Therapeutics, Inc.

### Notes to Consolidated Financial Statements (Continued)

December 31, 2012

### 2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES (Continued)

#### **Use of Estimates**

The preparation of the Company's consolidated financial statements requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts or revenue and expenses during the reporting period.

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

### **3. RECENT ACCOUNTING PRONOUNCEMENTS**

In May 2011, in an effort to assist in the convergence of U.S. GAAP and International Financial Reporting Standards ("IFRS"), the Financial Accounting Standards Board ("FASB") issued an Accounting Standards Update related to "Fair Value Measurements: Amendments to Achieve Common Fair Value Measurements and Disclosure Requirements in U.S. GAAP and IFRSs." The standard expands existing disclosure requirements for fair value measurements and makes certain other amendments, including a requirement to categorize, by level in the fair value hierarchy, items that are required to be disclosed, but not measured, at fair value. The standard is effective for fiscal years, and interim periods within those years, beginning after December 15, 2011, and should be applied prospectively. The Company adopted this standard as of January 1, 2012 and its adoption did not have a material effect on its consolidated financial statements.

### 4. COLLABORATION AGREEMENTS

#### Taiho Pharmaceutical Co., Ltd.

In October 2003, the Company entered into a license, research and development collaboration agreement with Taiho Pharmaceutical Co. Ltd. (Taiho) for mocetinostat, its clinical candidate, and its small molecule HDAC inhibitor program for oncology for Japan, South Korea, Taiwan, and China. Under the terms of the agreement, the Company received an up-front license fee of \$1.0 million, contract research funding of \$3.9 million and equity investment of \$2.7 million. In addition, the Company received \$5.4 million for preclinical and clinical funding through January 2006 and \$2.0 million for milestone payments in 2006 resulting in total proceeds of \$12.3 million relating to licensing and research and development activities and \$2.7 million relating to equity investment. In addition, the Company may receive milestone payments based on successful development, regulatory approval, and commercialization of an HDAC oncology product, and will receive royalties based on sales of HDAC oncology products in these territories as a percentage of annual net sales, which percentage is in the mid-single digit to mid-teen percent range, depending upon the total dollar amount of annual net sales, subject to reduction by a percentage in the range of 20-30% in the event a generic competitor is introduced in a particular market, other than in China. The term of the agreement will, on a country-by-country basis, continue until expiration of the last to expire issued patent, or ten years after the first commercial sale in Japan. Additionally, Taiho has a unilateral right to terminate the agreement for any reason with 30 days written notice, and we have a unilateral right to terminate the agreement if Taiho fails to make an undisputed payment. An arbitrator may terminate the agreement for a breach of obligations if such breach has remained uncured for 90 days. There was no revenue recognized from this agreement in 2012. In the year ended December 31, 2011 the Company recognized the

### Mirati Therapeutics, Inc.

Notes to Consolidated Financial Statements (Continued)

December 31, 2012

### 4. COLLABORATION AGREEMENTS (Continued)

remaining deferred revenue of \$420 thousand to license and up-front fees as the Company determined to stop the development of mocetinistat in 2011 which ended its substantive obligations in connection to this program.

#### Otsuka Pharmaceutical Co. Ltd.

On March 25, 2008, the Company entered into a worldwide research collaboration and license agreement with Otsuka Pharmaceutical Co. Ltd. (Otsuka) for the development of novel, small molecule, kinase inhibitors for local delivery and treatment of ocular diseases, excluding cancer. The Company was responsible for the design, characterization and initial screening of kinase inhibitors and determining which compounds to synthesize. Otsuka was responsible for funding efficacy and toxicity studies, as well as preclinical and clinical development of compounds. Otsuka is also responsible for the global commercialization of any resulting product. Under the terms of the agreement, the Company received an up-front license fee of \$2.0 million. There was no revenue recognized from this agreement in 2012. The Company may receive up to \$50.5 million based on successful development, regulatory, commercialization and sales milestones and will receive royalties as a percentage of annual net sales, which percentage is in the mid-single digit to mid-teen percent range dependent upon the total dollar amount of annual net sales, subject to a reduction by a percentage in the range of 40-50% in the event a generic competitor is introduced in a particular market, or intellectual property protection in a particular market does not exist or expires.

The Company may receive aggregate milestone payments of up to \$50.5 million under this agreement as follows: \$7.5 million relates to development activities, \$22.0 million relates to the completion of regulatory approvals and \$21.0 million relates to the achievement of certain sale goals.

Otsuka provided the Company with \$1.9 million in research funding for the initial 18 months of the research collaboration which was then extended on three occasions: September 10, 2009; April 23, 2010 and June 30, 2010. The research component of the agreement ended on June 30, 2011, subsequent to which the Company no longer has any significant ongoing obligations. In 2011, as the Company determined that its substantive performance obligations under the agreement ceased when the research component of the agreement ended on June 30, 2011, the Company accelerated the recognition of the remaining unamortized balance of \$1.7 million associated with the up-front license fee in the year ended December 31, 2011. The Company received a total of \$4.5 million in research funding from the research component of this agreement. In October 2009, Otsuka made, in relation to the terms of the agreement, a \$1.5 million equity investment in the Company's shares of common stock at a share price of CND\$21.30 (or \$20.27, as converted) which was a 20% premium over the five-day volume-weighted average closing price at the date of the transaction. Total proceeds in connection with this agreement, included research funding of \$6.5 million and the equity investment of \$1.5 million. On June 30, 2010, the collaboration agreement was amended to, among certain other changes, provide Otsuka the rights to synthesize a limited number of compounds predetermined by the Company. A lead molecule was selected in June 2011 for further development. Otsuka is currently advancing the lead compound through late preclinical development. The duration of the agreement is subject to future events. The term of the agreement will, on a country-by-country basis, continue until expiration of the last to expire issued patent, or if no patent has issued in such country, then 12 years after the first sale of a licensed product by Otsuka. Otsuka has a unilateral right to terminate the agreement for any reason with 90 days written notice and either party may

### Mirati Therapeutics, Inc.

#### Notes to Consolidated Financial Statements (Continued)

December 31, 2012

### 4. COLLABORATION AGREEMENTS (Continued)

terminate the agreement for a breach of obligations of the other party if such breach has remained uncured for 120 days (or 30 days for a breach of payment). Termination of Otsuka's Rights, in the event of a breach by Otsuka in one of its territories, will not affect its rights in non-breached territories.

#### **EnVivo Pharmaceuticals**

In March 2004, the Company entered into a proof of concept and option agreement with EnVivo Pharmaceuticals, Inc. (EnVivo) focusing on the treatment and prevention of neurodegenerative diseases, to exploit its HDAC inhibitors in diseases such as Huntington's, Parkinson's, and Alzheimer's. On February 7, 2005 the Company signed an exclusive research, collaboration and license agreement. During the year ended December 31, 2005, EnVivo paid the Company \$600 thousand for research, plus a \$500 thousand license fee for a total of \$1.1 million. As part of this agreement, EnVivo received a warrant to purchase 1,050 shares of common stock of the Company at an exercise price of CND\$214.30 (or \$170.62, as converted). The warrant expired on March 4, 2007. On February 6, 2008, the Company exercised its right to opt-out of the program. As a result, the Company has granted EnVivo exclusive rights to its HDAC inhibitors for neurodegenerative diseases and the Company ceased research and development funding for this program. The Company will receive royalties equal to a single digit percentage of net sales of any approved compound and will share in any sublicense income from future partnerships that EnVivo may enter into. The duration of the agreement terminates upon mutual agreement by the parties or when no product is under development or being commercialized. We did not recognize any revenue in connection with this agreement in either 2012 or 2011. We do not have any significant ongoing obligations in connection with this agreement.

### 5. CASH AND CASH EQUIVALENTS

	DECEMBER 31,				
	2012			2011	
	(in thousands)				
Cash at bank and on hand	\$	2,823	\$	1,287	
Bankers' acceptances		1,369		5,501	
Treasury bills		5,026		492	
Promissory notes		6,020		639	
Commercial papers		753		1,963	
Term deposit notes		2,714			
		18,705		9,882	
Less: restricted cash equivalents		(302)			
	\$	18,403	\$	9,882	



### Mirati Therapeutics, Inc.

Notes to Consolidated Financial Statements (Continued)

December 31, 2012

# 6. MARKETABLE SECURITIES AND TERM DEPOSITS

		DECEM 2012 (in thou	2	2011
Bankers' acceptances issued in Canadian currency, earning interest at 1.20% (1.15% in 2011) and maturing on February 19, 2013 (on various dates from May 28, 2012 to August 29, 2012 in 2011)	\$	72	\$	644
Commercial papers issued in Canadian currency, earning interest at rates ranging from 1.01% to 1.12% (0.85% to 1.01% in 2011) and maturing on various dates from February 21, 2013 to May 14, 2013 (March 29, 2012 to	Ψ	5.026	φ	
April 12, 2012 in 2011) Treasury bills issued in Canadian currency, earning interest at rates ranging from 0.85% to 0.90% and maturing		5,020		1,081
on various dates from January 18, 2012 to June 13, 2012				2,282
Guaranteed investment certificates issued in Canadian currency, earning interest at rates ranging from 1.15% to 1.35% (1.25% to 1.30% in 2011) and maturing on various dates from January 7, 2013 to September 16, 2013				
(April 12, 2012 to November 26, 2012 in 2011)		6,518		12,840
Term deposits issued in Canadian currency, earning interest at rates ranging from 1.30% to 1.33% (1.22% to 1.30% in 2011) and maturing on various dates from March 18, 2013 to April 15, 2013 (January 11, 2012 to				
February 1, 2012 in 2011)		7,036		2,360
		18,652		19,207
Less restricted marketable securities		(72)		(644)
	\$	18,580	\$	18,563

### 7. RESTRICTED CASH EQUIVALENTS AND MARKETABLE SECURITIES

In connection with obligations arising from an arrangement that became effective on May 19, 2010, the Company has a a letter of guarantee that is collateralized by a charge on a specific security in the amount of \$302 thousand and \$590 thousand included in restricted cash equivalents and marketable securities at December 31, 2012 and 2011, respectively.

The Company has established a letter of guarantee in relation to credit limits on its credit cards that is collateralized by a charge on a specific security in the amount of \$72 thousand and \$54 thousand included in restricted cash equivalents and marketable securities at December 31, 2012 and 2011, respectively.



# Mirati Therapeutics, Inc.

### Notes to Consolidated Financial Statements (Continued)

#### December 31, 2012

# 7. RESTRICTED CASH EQUIVALENTS AND MARKETABLE SECURITIES (Continued)

Restricted cash equivalents and marketable securities are comprised of cash equivalents and marketable securities as follows (in thousands):

	DECEMBER 31,				
	2	012	2	011	
Cash equivalents	\$	302	\$		
Marketable securities		72		644	
Less:		374		644	
Current portion		(302)		(295)	
	\$	72	\$	349	

# 8. INTEREST AND OTHER RECEIVABLES

	DECEMBER 31,					
	20	)12	2	011		
	(in thousands)					
Other receivables	\$	425	\$	60		
Interest receivable		82		112		
	\$	507	\$	172		

# 9. OTHER CURRENT ASSETS

	DECEMBER 3 2012 2012				
	(in thousand				
Refundable research and development tax credits	\$	593	\$	1,104	
Commodity taxes		165		176	
Prepaid expenses		779		268	
	\$	1,537	\$	1,548	

Research and development tax credits include a receivable of \$311 thousand from 9222-9129 Québec Inc. at December 31, 2011.

### **10. SECURITY DEPOSITS**

Security deposits were \$67 thousand and \$54 thousand at December 31, 2012 and 2011, respectively and are comprised of cash held in escrow that serves to guarantee certain obligations reflected in an employment contract and deposits issued to the landlords in Canada and the United States.

### Mirati Therapeutics, Inc.

Notes to Consolidated Financial Statements (Continued)

December 31, 2012

### **11. PROPERTY AND EQUIPMENT**

	<b>DECEMBER 31,</b>					
	2012			2011		
	(in thousands)					
Computer equipment	\$	1,421	\$	1,324		
Office and other equipment		89		66		
Laboratory equipment		1,794		1,769		
Leasehold improvements		56		52		
		3,360		3,211		
Less: Accumulated depreciation		(3,027)		(2,992)		
	\$	333	\$	219		

Depreciation expenses of \$97 thousand and \$26 thousand were included in research and development expenses and in general and administrative expenses, respectively, for the year ended December 31, 2012. Depreciation expenses of \$186 thousand and \$11 thousand were included in research and development expenses and in general and administrative expenses, respectively for the year ended December 31, 2011.

### 12. ACCOUNTS PAYABLE AND ACCRUED LIABILITIES

	<b>DECEMBER 31,</b>				
	2012	2	2011		
	(in thousands)				
Accounts payable	\$ 1,7	752 \$	767		
Accrued expenses	2,0	586	2,457		
Accrued compensation and benefits	8	334	525		
	\$ 5,2	272 \$	3,749		

### 13. STOCKHOLDERS' EQUITY

#### Warrants issued and outstanding

		Exercise		Number of
Issue date	Expiry date	p	orice	warrants
April 4, 2011	April 4, 2016	\$	7.71	1,655,314
November 21, 2012	November 21, 2017	\$	8.73	1,078,145

On November 21, 2012, the Company completed a private placement resulting in gross proceeds of \$26.1 million. Under the terms of the offering the Company issued a total of 3,593,819 units at a subscription price of CND\$7.25 (or \$7.28, as converted), each unit consisting of one share of common stock and thirty one-hundredths (0.30) of a common stock purchase warrant, exercisable for a period of five years from the date of issuance at an exercise price of CND\$8.70 (or \$8.73, as converted) (representing 120% of the subscription price). The issuance costs of the units amounted to \$1.3 million. The net proceeds were allocated to common stock and warrants based on their relative fair values.

### Mirati Therapeutics, Inc.

### Notes to Consolidated Financial Statements (Continued)

#### December 31, 2012

### 13. STOCKHOLDERS' EQUITY (Continued)

The fair value of the warrants issued on November 21, 2012 was estimated at the date of grant using the Black-Scholes option pricing model and the following assumptions: weighted average risk-free interest rate of 1.34%; dividend yield of nil; volatility factor of 115.5% and the expected life of the warrants of five years. The fair value allocated to the warrants amounted to \$4.9 million and \$19.9 million was recorded as an increase to common stock, net of costs.

On August 16, 2012, 9,654 common stock purchase warrants issued on April 4, 2011 in conjunction with the private placement were exercised. The number of shares issued in the cashless exercise transaction consisted of 5,653 shares of common stock. The fair value of the exercised warrants estimated at the date of grant using the Black-Scholes option pricing model was \$36.

On April 4, 2011, the Company completed a private placement resulting in gross proceeds of \$35.7 million. Under the terms of the offering, the Company issued 5,549,895 units including 123,121 units relating to the conversion of the convertible debt, which was issued on March 24, 2011 and which converted automatically on closing, at a subscription price of CND\$6.22 (or \$6.42, as converted); each unit consisting of one share of common stock and thirty one-hundredths (0.30) of a share of common stock purchase warrant, exercisable for a period of five years from the date of issuance at an exercise price of CND\$7.46 (or \$7.71 as converted) (representing 120% of the market price). The issuance costs of the units amounted to \$2.0 million. The net proceeds were allocated to common stock and warrants based on their relative fair values.

The fair value of the warrants issued on April 4, 2011 was estimated at the date of grant using the Black-Scholes option pricing model and the following assumptions: weighted average risk-free interest rate of 2.76%; dividend yield of nil; volatility factor of 86.2% and the expected life of the warrants of five years. The fair value allocated to the warrants amounted to \$6.2 million and \$27.4 million was recorded as an increase to common stock.

#### Stock-based compensation plan

The Company has in place a stock option plan (the "Plan") for the benefit of employees, directors, officers and consultants of the Company, which was amended by resolution of the shareholders of the Company on September 17, 2002, April 23, 2004, April 19, 2007, June 14, 2011 and most recently on June 27, 2012. The Plan was amended to increase the authorized share options available to be purchased to 700,000 from 240,000. As of December 31, 2012, there were 137,628 stock options available to be issued.

Most options vest over a period of four years, 20% on the date of award and 20% on each of the next four anniversary dates. The vesting period of the stock options is at the discretion of the Company's Board of Directors. The exercise price of any option granted under the Plan is based on the fair market value of common stock, determined by the closing sale price of the shares of common stock on the Toronto Stock Exchange, on the day before the stock options are granted, or if no sale is reported on that day, the "Market price" shall be deemed to be the volume weighted average trading price for the shares of common stock for the five days preceding the date of grant during which the shares of common stock were traded. The term of an option will not exceed ten years from the date of the grant and all options awarded after March 2005 have a five-year term.

### Mirati Therapeutics, Inc.

#### Notes to Consolidated Financial Statements (Continued)

#### December 31, 2012

### 13. STOCKHOLDERS' EQUITY (Continued)

The changes to the number of stock options granted by the Company and their weighted average exercise price are as follows:

	2012			2011			
		W	eighted		Weighted		
	Number of	average exercise		8		average exercise	
	options	price		orice options		price	
Balance, beginning of year	177,364	\$	26.50	43,036	\$	130.00	
Granted	532,304		11.00	157,333		17.50	
Forfeited	(147,112)		15.00	(20,251)		145.00	
Expired	(2,741)		167.00	(2,754)		181.50	
Exercised							
Balance, end of year	559,815		15.00	177,364		26.50	
Options exercisable, end of year	159,017	\$	22.50	59,454	\$	47.50	

The Company recorded stock-based compensation expense of \$0.8 million and \$1.2 million in research and development expenses and general and administrative expenses, respectively during the year ended December 31, 2012. The Company recorded stock-based compensation expense of \$0.3 million and \$0.7 million in research and development expenses and general and administrative expenses, respectively during the year ended December 31, 2012.

In the years ended December 31, 2012 and 2011, no stock-based compensation expense was capitalized and there were no recognized tax benefits associated with the stock-based compensation charge.

The fair value of options granted is estimated at the date of grant using the Black-Scholes option pricing model and the following assumptions:

	YEAR EN	YEAR ENDED			
	DECEMB	ER 31,			
Weighted average	2012	2011			
Risk-free interest rate	1.18%	2.04%			
Dividend yield	0%	0%			
Volatility factor	116.43%	116.79%			
Expected life in years	4.42	4.27			

The estimated fair value of the options is amortized to expense over the option's vesting period. The weighted average fair value of stock options granted under the Black-Scholes option pricing model and the above assumptions amounted to \$8.50 and \$13.50 in the year ended December 31, 2012 and 2011, respectively. The expected life of the options is based on historical data and current expectation and is not necessarily indicative of exercise patterns that may occur. Volatility is determined based on historical share price history, over a period similar to the expected life of the options, which may also not be necessarily

### Mirati Therapeutics, Inc.

### Notes to Consolidated Financial Statements (Continued)

December 31, 2012

### 13. STOCKHOLDERS' EQUITY (Continued)

indicative of the actual outcome. The risk-free interest rate is the rate for periods equal to the expected term of share option based on the Canadian Treasury yield in effect at the time of grant.

				Weighted-
				Average
		Wei	ghted-	Remaining
		Av	erage	Contractual
Vested and unvested expected to vest at		Ex	ercise	Term (in
December 31, 2012	Options	Price		years)
Outstanding at December 31, 2012	559,815	\$	15.00	4.34
Exercisable at December 31, 2012	159,017		22.50	3.94

The total compensation cost not yet recognized as of December 31, 2012 related to non-vested option awards was \$2.4 million which will be recognized over a weighted-average period of 2.13 years.

### **14. TAXATION**

#### Tax expense

The income tax expense reported differs from the amount of the tax expense (recovery) computed by applying Canadian federal and the applicable provincial statutory rates to loss before income taxes. The Canadian combined statutory rates were 26.90% in 2012 and 28.40% in 2011. The reasons for the differences and the related tax effects are as follows (in thousands):

	YEAR ENDED DECEMBER 31, 2012 2011		
Statutory federal and provincial taxes	\$	(5,446)	\$ (2,777)
Increase (decrease) in taxes recoverable resulting from:			
Effect of change in valuation allowance		5,145	2,829
Non-deductible stock-based compensation		539	267
Non-deductible expenses for tax purposes		3	2
Tax credits not taxable in Quebec		(70)	(106)
Share issue costs		(183)	(112)
Tax benefits on capitalized expenses		(1)	(24)
Effect of foreign jurisdiction tax expense		39	
Other differences		13	(79)
Income tax expense	\$	39	\$
	I	F-20	

## Mirati Therapeutics, Inc.

#### Notes to Consolidated Financial Statements (Continued)

#### December 31, 2012

#### 14. TAXATION (Continued)

The provision for the income tax expense, which relates to the United States, are as follows and relate to the wholly-owned subsidiary MethylGene US Inc. (in thousands):

	YEAR ENDED DECEMBER 31,			
	2012	2011		
Current income tax expense				
Current period	\$ 39	\$		
Income tax expense	\$ 39	\$		

## **Deferred** tax

Deferred tax relates to the following (in thousands):

	<b>DECEMBER 31,</b>				
Deferred tax asset		2012		2011	
Assets					
Tangible and intangible depreciable assets	\$	853	\$	833	
Inventory		757		474	
Provisions		30		8	
Financing fees		628		414	
Net operating loss carry forwards		6,883		3,490	
Scientific research and experimental development expenditures		4,245		2,406	
Deferred tax assets		13,396		7,625	
Valuation allowance		(13,396)		(7,625)	
Net deferred tax assets	\$		\$		

Total valuation allowance increased by \$5.8 million for the year ended December 31, 2012. The Company has evaluated positive and negative evidence bearing upon the ability of its deferred tax assets to be realized. The Company has determined that it is more likely than not that it will not recognize the benefits of its federal and provincial deferred tax assets and, as a result, has established a full valuation allowance against its deferred tax assets as of December 31, 2012.

For Canadian I.C. Federal income tax purposes, the Company's Canadian federal scientific research and experimental development expenditures amounted to \$15.1 million and \$8.5 million for the years ended December 31, 2012 and 2011, respectively and for provincial income tax purposes amounted to \$16.6 million and \$9.4 million for the years ended December 31, 2012 and 2011, respectively. These expenditures are available to reduce future taxable income and have an unlimited carry forward period. Scientific research and development expenditures are subject to verification by the taxation authorities, and accordingly, these amounts may vary by a material amount.

The Company's net operating loss carry forwards ("NOLs") for Canadian federal income tax purposes, were \$25.5 million and \$13.0 million at December 31, 2012 and 2011, respectively. The Company's NOLs were

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## Mirati Therapeutics, Inc.

#### Notes to Consolidated Financial Statements (Continued)

#### December 31, 2012

#### 14. TAXATION (Continued)

\$25.7 million and \$13.1 million at December 31, 2012 and 2011, respectively, for provincial tax purposes.

The NOLs are available to offset future taxable income from Canadian federal and provincial tax sources and the tax benefits of which have not been recognized in the consolidated financial statements. The NOLs expire as follows (in thousands):

	Fe	Federal		vincial
Expires in:				
2030	\$	5,907	\$	5,984
2031		7,059		7,066
2032		12,547		12,632
	\$	25,513	\$	25,682

The Company's Canadian operations have been audited for provincial tax purposes up to and including December 31, 2009. For Canadian federal tax purposes, the Company remains subject to audit for the December 31, 2008 and subsequent taxation years. Where taxation years remain open, the Company considers it reasonably possible that issues may be raised or tax positions agreed to with the taxation authorities, which may result in increases or decreases of the balance of non-refundable ITCs and NOLs. However, an estimate of such increases and decreases cannot be currently made.

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

	FEDERAL DECEMBER 31,			PROVINCIAL DECEMBER 31,			
2	2012 2011		2012 2		20	2011	
\$	157	\$	155	\$	3	\$	1
	2		2		2		2
\$	159	\$	157	\$	5	\$	3
	D 2	DECE 3 2012 \$ 157 2	DECEMB 31, 2012 2 \$ 157 \$ 2	DECEMBER 31, 2012 2011 \$ 157 \$ 155 2 2	DECEMBER         DI           31,         2012         2011         20           \$ 157         \$ 155         \$           2         2         2	DECEMBER         DECENTION           31,         31           2012         2011         2012           \$ 157         \$ 155         \$ 3           2         2         2	DECEMBER         DECEMBER           31,         31,           2012         2011         2012         20           \$ 157         \$ 155         \$ 3         \$           2         2         2         2

The Company had no accrual for interest or penalties on tax matters as at December 31, 2012 and 2011 and the Company had no ongoing tax audits as of December 31, 2012.

## **15. INVESTMENT TAX CREDITS**

The Company is eligible to claim Canadian federal and provincial ITCs for eligible scientific research and development expenditures. The Company records ITCs based on management's best estimates of the amount to be recovered and ITCs claimed are subject to audit by the taxation authorities and accordingly, may vary by a material amount.

## Mirati Therapeutics, Inc.

#### Notes to Consolidated Financial Statements (Continued)

December 31, 2012

## 15. INVESTMENT TAX CREDITS (Continued)

The Company recorded provincial refundable ITCs as a reduction of research and development expenditures of \$1.7 million (including a \$1.1 million favorable adjustment resulting from a statutory audit), and \$0.9 million for the years ended December 31, 2012 and 2011, respectively.

The Company's non-refundable Canadian federal ITCs amount to \$3.0 million and \$1.8 million at December 31, 2012 and 2011, respectively, and relate to scientific research and development expenditures, which may be utilized to reduce Canadian federal income taxes payable in future years. The benefits of the non-refundable Canadian federal ITCs have not been recognized in the financial statements and will be recorded as reduction of tax expense when realized.

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

	 FEDERAL ITC		
Expires in:			
2030	\$ 760		
2031	1,000		
2032	1,266		
	\$ 3,026		

## **16. NET LOSS PER SHARE**

#### **Basic and diluted**

Net loss per share is calculated by dividing the net loss of the Company by the weighted average number of shares of common stock outstanding during the year.

	YEAR ENDED DECEMBER 31,					
		2012 20		2012 2011		2011
	(in thousands except for					
		sha	re			
	aı	nd per shan	e ai	mounts)		
Net loss and comprehensive loss for the year	\$	(20,286)	\$	(9,778)		
Weighted average number of shares of common stock		6,762,985		4,944,184		
Basic and diluted net loss per share	\$	(3.00)	\$	(1.98)		

Common stock equivalents from warrants and options were excluded from weighted average number of shares of common stock outstanding for the purpose of calculating diluted net loss per share, because the effect is anti-dilutive.

## Mirati Therapeutics, Inc.

#### Notes to Consolidated Financial Statements (Continued)

December 31, 2012

## **17. COMMITMENTS AND CONTINGENCIES**

#### (i) Operating leases

The Company is committed under operating leases for the lease of its premises and certain office equipment. Future minimum annual payments required over the next three years are as follows (in thousands):

	Minii Lea	
	Payn	nents
2013	\$	203
2014		153
2015		17
	\$	373

During the year ended December 31, 2011, the Company entered into a lease agreement with the landlord for a 36-month term from September 1, 2011 to August 31, 2014, which included an element of free rent that will be amortized over the term of the lease. Expense of \$85 thousand and \$28 thousand was recorded in the consolidated statement of operations and comprehensive loss for the years ended December 31, 2012 and 2011, respectively.

The Company entered into a lease agreement in Princeton, New Jersey regarding MethylGene US Inc. The lease term is 36 months which started May 1, 2012 and will end April 30, 2015.

Lease expense was \$305 thousand and \$190 thousand for the years ended December 31, 2012 and 2011, respectively.

#### (ii) Research and development contracts

The Company is committed to several ongoing clinical development supplier contracts. Future commitments relating to these contracts at December 31, 2012 amounted to approximately \$392 thousand which is expected to be paid in 2013.

#### (iii) Other guarantees

The Company regularly enters into agreements with third parties that include indemnification provisions that are customary in the industry. These guarantees generally require the Company to compensate the other party for certain damages and costs incurred as a result of third-party intellectual property claims or damages arising from the use of the intellectual property. In some cases, the maximum potential amount of future payments that could be required under these indemnification provisions is unlimited. These indemnification provisions generally survive termination of the underlying agreement. The nature of the intellectual property indemnification obligations prevents the Company from making a reasonable estimate of the maximum potential amount it could be required to pay. The Company has granted indemnifications to corporate partners, contract research organizations, contract manufacturers and clinical trial sites and others.

## Mirati Therapeutics, Inc.

Notes to Consolidated Financial Statements (Continued)

December 31, 2012

## 17. COMMITMENTS AND CONTINGENCIES (Continued)

Historically, the Company has not made any indemnification payments under such agreements and no amount has been accrued in the accompanying consolidated financial statements with respect to these indemnification obligations.

#### **18. SEGMENT INFORMATION**

The Company operates in a single business segment from one facility in Canada and one in the USA.

The Company's revenue was derived from collaboration partners located as follows (in thousands):

	YEAR ENDED DECEMBER 31,			
	2012	2	2011	
United States	\$	\$	3	
Japan			3,141	
	\$	\$	3,144	

The Company's long lived assets are located as follows (in thousands):

	YEAR ENDED DECEMBER 31,				
	20	2012		011	
Canada	\$	299	\$	219	
United States		34			
	\$	333	\$	219	

#### **19. FAIR VALUE OF FINANCIAL INSTRUMENTS**

The following tables present information about assets that are measured at fair value on a recurring basis for the periods presented and indicates the fair value hierarchy of the valuation techniques we utilized to determine such fair value.

In general, fair values determined by Level 1 inputs utilize quoted prices (unadjusted) in active markets for identical assets or liabilities.

Fair values determined by Level 2 inputs utilize data points that are observable such as quoted prices, interest rates and yield curves.

Fair values determined by Level 3 inputs are unobservable data points for the asset or liability, and include situations where there is little, if any, market activity for the asset or liability.

## Mirati Therapeutics, Inc.

#### Notes to Consolidated Financial Statements (Continued)

#### December 31, 2012

## 19. FAIR VALUE OF FINANCIAL INSTRUMENTS (Continued)

There were no transfers in or out of Level 1 or Level 2 measurements for the periods presented as follows (in thousands):

	DEC	EMBER					
		31,	LEVEL	L	EVEL	LEV	<b>'EL</b>
	:	2012	1		2	3	5
Cash equivalents	\$	15,580	\$	\$	15,580	\$	
Marketable securities and term deposits	\$	18,580	\$	\$	18,580	\$	
Restricted cash equivalents and marketable securities	\$	374	\$	\$	374	\$	

	DEC	EMBER					
		31,	LEVEL	L	EVEL	LEVE	L
	:	2012	1		2	3	
Cash equivalents	\$	8,595	\$	\$	8,595	\$	
Marketable securities and term deposits	\$	18,563	\$	\$	18,563	\$	
Restricted cash equivalents and marketable securities	\$	644	\$	\$	644	\$	
20. CONCENTRATION OF CREDIT RISK							

The maximum exposure to credit risk of the Company at December 31, 2012 is the carrying value of its cash and cash equivalents, marketable securities, restricted cash equivalents and marketable securities, interest receivable, other receivables and security deposits. The Company has an investment policy that monitors the safety and preservation of investments made, which requires them to be highly rated and which limits the amount invested in any one issuer. The investments are reviewed quarterly by the Audit Committee.

The Company also manages credit risk by maintaining bank accounts with reputable banks and financial institutions and investing only in investments from banking, governmental or highly rated companies with securities that are traded on active markets and are capable of immediate liquidation, subject to some minor market price variations upon sale.

Cash and cash equivalents and restricted cash are comprised of bankers' acceptances, treasury bills, commercial papers, promissory notes and term deposits at December 31, 2012 (bankers' acceptances, treasury bills, commercial papers and promissory notes at December 31, 2011). Cash and cash equivalents and restricted cash as of December 31, 2012 and 2011are held in two Canadian chartered banks and are as follows (in thousands):

	<b>DECEMBER 31,</b>					
		2012	,	2011		
Cash and cash equivalents	\$	18,403	\$	9,882		
Restricted cash equivalents and marketable securities		374		644		
	\$	18,777	\$	10,526		

Management has determined that other receivables are collectible and has not recorded a provision against these amounts.

## Mirati Therapeutics, Inc.

#### Notes to Consolidated Financial Statements (Continued)

#### December 31, 2012

## 20. CONCENTRATION OF CREDIT RISK (Continued)

The Company continues to have ongoing license and collaboration agreements with three partners. As per the term of these agreements there was no revenue or expenses with these partners in 2012. In 2011 two corporate partners accounted for 78% and 11%, of revenue and expenses, respectively.

## **21. SUBSEQUENT EVENTS**

For its financial statements as of December 31, 2012 and for the year then ended, the Company evaluated subsequent events through May 8, 2013, the date on which those financial statements were originally available to be issued.

The Company regularly reviews its functional currency. Based on a detailed analysis of projected expenses the Company has determined that it will transition to the United States dollar as its functional currency effective January 1, 2013.

Mirati was incorporated under the laws of the State of Delaware on April 29, 2013. The Company was created to enter into an arrangement agreement described below.

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

## Mirati Therapeutics, Inc. CONDENSED CONSOLIDATED BALANCE SHEETS (in thousands) (unaudited)

	Л	U <b>NE 30,</b>	DE	CEMBER 31,
	-	2013		2012
ASSETS		_010		
Current assets				
Cash and cash equivalents	\$	9,740	\$	18,403
Marketable securities		10,516		18,580
Restricted cash equivalents and marketable securities		285		302
Interest and other receivables		108		507
Other current assets		1,787		1,537
Total current assets		22,436		39,329
		22,430		39,329
Security deposits		101		67
Restricted cash equivalents and marketable securities		78		72
Property and equipment, net		425		333
Total assets	\$	23,040	\$	39,801
		,		
LIABILITIES AND STOCKHOLDERS' EQUITY				
Current liabilities				
Accounts payable and accrued liabilities		4,516		5,272
Current portion of other liability		68		68
Warrant liability		12,208		
Total current liabilities		16,792		5,340
Others High High		11		45
Other liability		11		45
Total liabilities		16,803		5,385
Stockholders' equity				
Preferred stock, \$0.001 par value, 10,000,000 shares authorized; none issued and outstanding at both June 30, 2013 and December 31, 2012				
Common stock, \$0.001 par value; 100,000,000 authorized; 9,957,725 issued and outstanding at both				
June 30, 2013 and December 31, 2012		10		10
Warrants		_0		11,153
Additional paid-in capital		154,469		154,224
Accumulated other comprehensive income		9,520		9,520
Accumulated deficit		(157,762)		(140,491)
Total stockholders' equity		6,237		34,416
Total Stockholdel's cylley		0,237		54,410
Total liabilities and stockholders' equity	\$	23,040	\$	39,801

See accompanying notes

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## Mirati Therapeutics, Inc. CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS (in thousands except for share and per share amounts) (unaudited)

		THREE M END JUNE	ED		HS ENDED E 30,
		2013	2012	2013	2012
Expenses					
Research and development, net	\$	4,510	\$ 3,652	\$ 9,985	\$ 5,856
General and administrative		2,382	1,082	4,906	2,302
Total operating expenses		6,892	4,734	14,891	8,158
Loss from operations		(6,892)	(4,734)	(14,891)	(8,158)
Other (expense) income, net		(1,079)	70	2,722	138
Loss before income taxes		(7,971)	(4,664)	(12,169)	(8,020)
Income tax expense		41	13	60	13
1					
Net loss and comprehensive loss	\$	(8,012)	\$ (4,677)	\$ (12,229)	\$ (8,033)
		(-)/			( ( ) ) = ( )
Basic and diluted net loss per share	\$	(0.81)	\$ (0.73)	\$ (1.23)	\$ (1.26)
	*	(0.01)	¢ (0.75)	÷ (1120)	¢ (1.20)
Weighted average number of shares used in computing net loss per					
share, basic and diluted		9,957,725	6,358,266	9,957,725	6,358,266
shure, busie and diluted		2,201,140	0,550,200	2,251,125	0,550,200

See accompanying notes

## Mirati Therapeutics, Inc. CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS (in thousands) (unaudited)

		SIX MONTHS ENDED JUNE 30,			
		2013	2	012	
Operating activities	đ	(12.220)	¢	(0.022)	
Net loss	\$	(12,229)	\$	(8,033)	
Non-cash adjustments reconciling net loss to operating cash flows		<i>.</i>		(0)	
Depreciation of property and equipment		64		68	
Stock-based compensation expense		245		652	
Change in fair value of warrant liability		(3,987)		10	
Lease incentive		(34)		43	
Changes in operating assets and liabilities		200		74	
Interest and other receivables		399		74	
Other current assets		(250)		290	
Accounts payable and accrued liabilities		(756)		(373)	
Cash flows used for operating activities		(16,548)		(7,279)	
Investing activities					
Purchases of property and equipment		(156)		(51)	
Purchases of marketable securities		(22,391)		(13,419)	
Security deposit		(34)		(15)	
Restricted cash equivalents and marketable securities		11			
Disposal and maturities of marketable securities		30,455		18,906	
Cash flows provided by investing activities		7,885		5,420	
Financing activities					
Costs of reorganization				(16)	
Cash flame used for first striction				(16)	
Cash flows used for financing activities				(16)	
Decrease in cash and cash equivalents		(8,663)		(1,875)	
Effect of exchange rate changes on cash and cash equivalents				44	
Cash and cash equivalents, beginning of period		18,403		9,882	
Cash and cash equivalents, end of period	\$	9,740	\$	8,051	

See accompanying notes

Mirati Therapeutics, Inc.

Notes to Consolidated Financial Statements

June 30, 2013 (unaudited)

## 1. DESCRIPTION OF BUSINESS

Mirati Therapeutics, Inc. ("Mirati" or the "Company") is a biopharmaceutical company and its primary business purpose is to develop and commercialize novel therapeutics for the treatment of patients with cancer.

The Company has a wholly owned subsidiary in Canada, Methylgene, Inc. ("Methylgene"). MethylGene's common stock was listed on the Toronto Stock Exchange from June 29, 2004 until July 26, 2013 under the ticker symbol "MYG". The Company also has an indirect, wholly-owned subsidiary, Methylgene US Inc., which was incorporated in Princeton, New Jersey on December 20, 2011 and started business activity in 2012. The Company's common stock has been listed on the NASDAQ Capital Market since July 15, 2013 under the ticker symbol "MRTX". The Company is a holding company with minimal assets other than the stock of its subsidiary in Canada, MethylGene Inc., and primarily conducts its operations through MethylGene and MethylGene US Inc. Refer to Note 2 under the heading Basis of Presentation and Going Concern Uncertainty for further discussion of the Company's corporate structure.

## 2. BASIS OF PRESENTATION AND GOING CONCERN UNCERTAINTY

The information contained herein has been prepared in accordance with instructions for Form 10-Q and Article 10 of Regulation S-X. The information as of June 30, 2013, and for the six months ended June 30, 2013 and 2012, is unaudited. In the opinion of management, the information reflects all adjustments necessary to make the results of operations for the interim periods a fair statement of such operations. All such adjustments are of a normal recurring nature. Interim results are not necessarily indicative of results for the full year. The consolidated balance sheet at December 31, 2012 has been derived from the audited consolidated financial statements at that date, but does not include all information and footnotes required by U.S. generally accepted accounting principles for complete financial statements. For more complete financial information, these financial statements should be read in conjunction with the audited consolidated financial statements included in Mirati's Registration Statement on Form 10 (No. 001-35921), originally filed with the Securities and Exchange Commission ("SEC") on May 10, 2013, as amended.

Mirati was incorporated under the laws of the State of Delaware on April 29, 2013. The Company was created to enter into an arrangement agreement with MethylGene described below.

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

Mirati Therapeutics, Inc.

Notes to Consolidated Financial Statements (Continued)

June 30, 2013 (unaudited)

#### 2. BASIS OF PRESENTATION AND GOING CONCERN UNCERTAINTY (Continued)

These financial statements have been prepared on a going concern basis, which contemplates the realization of assets and the settlement of liabilities in the normal course of business. The Company has incurred significant operating losses since inception and has relied on its ability to fund its operations through private and public equity financings and a debt financing. At June 30, 2013, the Company had \$20.6 million in cash, cash equivalents, marketable securities and restricted cash.

As of June 30, 2013, substantial doubt exists over the ability of the Company to continue as a going concern. The Company believes that its current cash and cash equivalents, marketable securities and restricted cash equivalents and marketable securities are sufficient to carry out its currently planned clinical development and operating plans into the second quarter of 2014, without considering potential future financing. The Company's cash, cash equivalents and marketable securities decreased by \$16.7 million in the six months ended June 30, 2013, reflecting an average rate of negative cash flow per month of approximately \$2.8 million. Excluding non-recurring costs associated with the previously described Arrangement, listing on the NASDAQ Capital Market and recent management changes of \$2.6 million, of which \$1.4 million relates to the Arrangement and NASDAQ listing, our cash, cash equivalents and marketable securities decreased by \$14.1 million in the six months ended June 30, 2013 reflecting an average rate of negative cash flow per month of approximately \$2.4 million. While the rate of future negative cash flow per month will vary due to the timing of expenses incurred and the programs that are funded, at the current rate of negative cash flow per month the Company believes that its current cash, cash equivalents and marketable securities will enable it to complete Phase 1 development of MGCD265, which if successful would enable it to enter Phase 2 development. The Company's future cash requirements could increase if it decides to expand research and development efforts beyond the currently planned development of MGCD265.

The Company has incurred operating losses in each year since its inception and expects to continue to incur operating losses into the foreseeable future as it advances the development of its product candidate MGCD265; evaluates opportunities for the potential initiation of further clinical development of mocetinostat; evaluates opportunities for the potential clinical development of MGCD516, and continues its research efforts. To fund future operations the Company will need to raise additional capital. The amount and timing of future funding requirements will depend on many factors including the timing and results of ongoing development efforts, the potential expansion of current development programs, potential new development programs and related general and administrative support. The Company anticipates that it will seek to fund its operations through public or private equity or debt financings or other sources, such as potential collaboration agreements. Additional financing may not be available to the Company on favorable terms, or at all. Although the Company has previously been successful in obtaining financing through its equity securities offerings, it may not be able to do so in the future. If the Company is not able to secure adequate additional financings it may be forced to make reductions in spending and/or liquidate assets where possible. Any of these actions could harm the Company's business and its results of operations.

These condensed interim consolidated financial statements do not reflect the adjustments that might be necessary to the carrying amount of reported assets, liabilities and expenses if the Company were unable to continue operations in accordance with the going concern assumption, and such adjustments could be material.

## Mirati Therapeutics, Inc.

#### Notes to Consolidated Financial Statements (Continued)

June 30, 2013 (unaudited)

#### 2. BASIS OF PRESENTATION AND GOING CONCERN UNCERTAINTY (Continued)

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

The Company has not early adopted any standard or amendment that has been issued but not yet effective.

## 3. SIGNIFICANT ACCOUNTING POLICIES

#### **Foreign Currency Transactions**

Foreign currency transactions are initially recorded by the Company using the exchange rates prevailing at the date of the transaction. At the balance sheet date, monetary assets and liabilities denominated in foreign currencies are translated at the period-end rates of exchange. Non-monetary assets and liabilities are translated at the historical exchange rates. Exchange gains and losses arising from the translation of foreign currency items are included in other (expense)/income in the consolidated statements of operations and comprehensive loss. The Company recognized net foreign exchange losses of \$754,000 and net foreign exchange gains of \$11,000 in other (expense)/income in the consolidated statement of operations and comprehensive loss for the three months ended June 30, 2013 and 2012, respectively. The Company recognized net foreign exchange losses of \$1.4 million and net foreign exchange gains of \$10,000 in other (expense)/income in the consolidated statement of operations and comprehensive loss for the six months ended June 30, 2013 and 2012, respectively.

#### **Reclassification of Warrants**

In 2011 and 2012, the Company issued common stock warrants in connection with the issuance of common stock through private placements with exercise prices denominated in Canadian dollars, referred to as the 2011 Warrants and 2012 Warrants, respectively. Upon the issuance of the 2011 and 2012 Warrants, the Company allocated the net proceeds to common stock and warrants based on their relative fair values, and calculated the fair value of the issued common stock warrants utilizing the Black-Scholes option-pricing model. The allocated fair value was then recorded as warrants within stockholders' equity on the consolidated balance sheet. The fair value was not remeasured in periods subsequent to the date of issuance.

The change in its functional currency to the U.S. dollar effective January 1, 2013 changed how the Company accounts for its warrants which have exercise prices denominated in Canadian dollars. Upon the change in functional currency, the Company classified these warrants as a current liability and recorded a warrant liability of \$16.2 million which represents the fair market value of the warrants at that date in accordance with Accounting Standards Codification, or ASC, 815, "*Derivatives and Hedging*". The initial fair value recorded as warrants within stockholders' equity of \$11.2 million was reversed. The change in fair value related to periods prior to January 1, 2013 of \$5.0 million was recorded as an adjustment to accumulated deficit. At each reporting period subsequent to January 1, 2013, the Company will adjust the fair value of the warrant liability and any corresponding increase or decrease to the warrant liability will be recorded as a component of other (expense)/income on the consolidated statement of operations and comprehensive loss. The estimated fair value is determined using the Black-Scholes option-pricing model based on the estimated value of the underlying common stock at the valuation measurement date, the remaining contractual term of the warrants, risk-free interest rates, expected dividends and expected volatility of the price of the underlying common stock. The fair value of the warrant liability was

## Mirati Therapeutics, Inc.

#### Notes to Consolidated Financial Statements (Continued)

June 30, 2013 (unaudited)

#### 3. SIGNIFICANT ACCOUNTING POLICIES (Continued)

\$12.2 million at June 30, 2013 and we recorded a loss of \$385,000 for the three months ended June 30, 2013 and a gain of \$4.0 million for the six months ended June 30, 2013 which is included in other (expense)/income in the consolidated statement of operations and comprehensive loss.

#### 4. COLLABORATION AGREEMENTS

#### Taiho Pharmaceutical Co., Ltd.

In October 2003, the Company entered into a license, research and development collaboration agreement with Taiho Pharmaceutical Co. Ltd. ("Taiho") pursuant to which the Company licensed rights to mocetinostat and its small molecule HDAC inhibitor program for oncology in Japan, South Korea, Taiwan, and China. Under the terms of the agreement, the Company received an up-front license fee of \$1.0 million, contract research funding of \$3.9 million and equity investment of \$2.7 million. In addition, the Company received \$5.4 million for preclinical and clinical funding through January 2006 and \$2.0 million for milestone payments in 2006 resulting in total proceeds of \$12.3 million relating to licensing and research and development activities and \$2.7 million relating to equity investment. In addition, the Company may receive milestone payments based on successful development, regulatory approval, and commercialization of an HDAC oncology product, and will receive royalties based on sales of HDAC oncology products in these territories as a percentage of annual net sales, which percentage is in the mid-single digit to mid-teen percent range, depending upon the total dollar amount of annual net sales, subject to reduction in the range of 20-30%, in the event a generic competitor is introduced in a particular market, other than in China. The term of the agreement will, on a country-by-country basis, continue until expiration of the last to expire issued patent, or ten years after the first commercial sale in Japan. Additionally, Taiho has a unilateral right to terminate the agreement for any reason with 30 days' written notice, and we have a unilateral right to terminate the agreement if Taiho fails to make an undisputed payment. An arbitrator may terminate the agreement for a breach of obligations if such breach has remained uncured for 90 days. The duration of the agreement is subject to future events. Termination can occur due to breach which is not cured within 30 days; due to insolvency; or when the royalty term for all licensed products ends. If mocetinostat development is restarted, both Taiho's and the Company's obligations in connection with this program would need to be evaluated and such obligations may continue unless the agreement is modified by the parties. There was no revenue recognized from this agreement in either the three or six months ended June 30, 2013 or 2012, respectively.

#### Otsuka Pharmaceutical Co. Ltd.

On March 25, 2008, the Company entered into a worldwide research collaboration and license agreement with Otsuka Pharmaceutical Co. Ltd. (Otsuka) for the development of novel, small molecule, kinase inhibitors for local delivery and treatment of ocular diseases, excluding cancer. The Company was responsible for the design, characterization and initial screening of kinase inhibitors and determining which compounds to synthesize. Otsuka was responsible for funding efficacy and toxicity studies, as well as preclinical and clinical development of compounds. Otsuka is also responsible for the global commercialization of any resulting product. Under the terms of the agreement, the Company received an up-front license fee of \$2.0 million. There was no revenue recognized from this agreement in either the three or six months ended June 30, 2013 or 2012, respectively. The Company may receive up to \$50.5 million based on successful development, regulatory, commercialization and sales milestones and will

Mirati Therapeutics, Inc.

Notes to Consolidated Financial Statements (Continued)

June 30, 2013 (unaudited)

#### 4. COLLABORATION AGREEMENTS (Continued)

receive royalties as a percentage of annual net sales, which percentage is in the mid-single digit to mid-teen percent range dependent upon the total dollar amount of annual net sales, subject to a reduction by a percentage in the range of 40-50% in the event a generic competitor is introduced in a particular market, or intellectual property protection in a particular market does not exist or expires.

The Company may receive aggregate milestone payments of up to \$50.5 million under this agreement as follows: \$7.5 million related to development activities, \$22.0 million related to the completion of regulatory approvals and \$21.0 million related to the achievement of certain sale goals.

Otsuka provided the Company with \$1.9 million in research funding for the initial 18 months of the research collaboration which was then extended on three occasions: September 10, 2009, April 23, 2010 and June 30, 2010. The research component of the agreement ended on June 30, 2011, subsequent to which the Company no longer has any significant ongoing obligations. In 2011, as the Company determined that its substantive performance obligations under the agreement ceased when the research component of the agreement ended on June 30, 2011, the Company accelerated the recognition of the remaining unamortized balance of \$1.7 million associated with the up-front license fee in the year ended December 31, 2011. The Company received a total of \$4.5 million in research funding from the research component of this agreement. In October 2009, Otsuka made, in relation to the terms of the agreement, a \$1.5 million equity investment in the Company's shares of common stock at a share price of CND\$21.30 (or \$20.75, as converted) in which was a 20% premium over the five-day volume-weighted average closing price at the date of the transaction. Total proceeds received as of June 30, 2013 in connection with this agreement, included research funding of \$6.5 million and equity investment of \$1.5 million. On June 30, 2010, the collaboration agreement was amended to, among certain other changes; provide Otsuka the rights to synthesize a limited number of compounds predetermined by the Company. A lead molecule was selected in June 2011 for further development. Otsuka is currently advancing the lead compound through late preclinical development. The duration of the agreement is subject to future events. The term of the agreement will, on a country-by-country basis, continue until expiration of the last to expire issued patent, or if no patent has issued in such country, then 12 years after the first sale of a licensed product by Otsuka. Otsuka has a unilateral right to terminate the agreement for any reason with 90 days written notice and either party may terminate the agreement for a breach of obligations of the other party if such breach has remained uncured for 120 days (or 30 days for a breach of payment). Termination can occur by a material breach by either party which is not cured within up to 120 days; or in the event Otsuka has not exercised its right to designate at least one Selected Compound (as defined in the agreement) during the Selection Period (as defined in the agreement).

## **EnVivo Pharmaceuticals**

In March 2004, the Company entered into a proof of concept and option agreement with EnVivo Pharmaceuticals, Inc. ("EnVivo") focusing on the treatment and prevention of neurodegenerative diseases, to exploit its HDAC inhibitors in diseases such as Huntington's, Parkinson's, and Alzheimer's. On February 7, 2005 the Company signed an exclusive research, collaboration and license agreement. During the year ended December 31, 2005, EnVivo paid the Company \$0.6 million for research, plus a \$0.5 million license fee for a total of \$1.1 million. As part of this agreement, EnVivo received a warrant to purchase 1,050 shares of common stock of the Company at an exercise price of CND\$214.30 (or \$171.55,

## Mirati Therapeutics, Inc.

#### Notes to Consolidated Financial Statements (Continued)

June 30, 2013 (unaudited)

#### 4. COLLABORATION AGREEMENTS (Continued)

as converted). The warrant expired on March 4, 2007. On February 6, 2008, the Company exercised its right to opt-out of the program. As a result, the Company has granted EnVivo exclusive rights to its HDAC inhibitors for neurodegenerative diseases and the Company ceased research and development activities for this program. The Company is entitled to receive royalties equal to a single digit percentage of net sales of any approved compound and will share in any sublicense income from future partnerships that EnVivo may enter into. The duration of the agreement is subject to future events. Either party can terminate the agreement due to a material breach by the other party that is not cured within 30 days or the other party's insolvency. The agreement will also terminate upon mutual agreement by the parties or when no product is under development or being commercialized. The Company did not recognize any revenue in connection with this agreement in either the three or six months ended June 30, 2013 or 2012. The Company does not have any significant ongoing development obligations in connection with this agreement.

## 5. CASH AND CASH EQUIVALENTS

	DECEMBER				
	JU	NE 30,	31,		
	,	2013	2012		
		(in th	ousands)		
Cash at bank and on hand	\$	2,333	\$ 2,823		
Bankers' acceptances		78	1,369		
Treasury bills		949	5,026		
Promissory notes		2,372	6,020		
Commercial papers		2,184	753		
Term deposit notes		1,902	2,714		
		9,818	18,705		
Less: restricted cash equivalents		(78)	(302)		
	\$	9,740	\$ 18,403		
			F-36		

## Mirati Therapeutics, Inc.

## Notes to Consolidated Financial Statements (Continued)

June 30, 2013 (unaudited)

## 6. MARKETABLE SECURITIES

	-	NE 30, 2013 (in th		CEMBER 31, 2012 ands)
Bankers' acceptance issued in Canadian currency, earning interest at a rate of 1.07% (1.20% in 2012) and	¢	295	¢	70
maturing on August 23, 2013 (February 19, 2013 in 2012) Commercial paper issued in Canadian currency, earning interest at a rate of 1.03% (1.01% to 1.12% in	\$	285	\$	72
2012) and maturing on August 15, 2013 (February 21, 2013 to May 14, 2013 in 2012)		1,141		5,026
Treasury bills issued in Canadian currency, earning interest at rates ranging from 0.98% to 1.06% and maturing on July 31, 2013 and September 4, 2013		1,959		
Guaranteed investment certificates issued in Canadian currency, earning interest at rates ranging from 1.15% to 1.30% (1.15% to 1.35% in 2012) and maturing on various dates from September 9, 2013 to		,		
February 7, 2014 (January 7, 2013 to September 16, 2013 in 2012)		6,228		6,518
Term deposits issued in Canadian currency, earning interest at a rate of 1.36% (1.30% to 1.33% in 2012)				
and maturing on September 13, 2013 (March 18, 2013 to April 15, 2013 in 2012)		1,188		7,036
		10,801		18,652
Less restricted marketable securities		(285)		(72)
	\$	10,516	\$	18,580

## 7. INTEREST AND OTHER RECEIVABLES

	JUNI 20	CCEMBER 31, 2012 ands)			
Other receivables	\$	`	10US \$	<b>4</b> 25	
Interest receivable	Ψ	42	ψ	82	
	\$	108	\$	507	
					F-37

#### Mirati Therapeutics, Inc.

Notes to Consolidated Financial Statements (Continued)

June 30, 2013 (unaudited)

#### 8. OTHER CURRENT ASSETS

	DECEMBER			
	<b>JUNE 30,</b>			31,
	2013			2012
		(in tl	ious	ands)
Refundable research and development tax credits	\$	1,030	\$	593
Commodity taxes		328		165
Prepaid expenses		429		779
	\$	1,787	\$	1,537

## 9. ACCOUNTS PAYABLE AND ACCRUED LIABILITIES

		DECEMBER				
	JUNE 30, 2013		<b>JUNE 30,</b>		31,	
				2012		
	(in thousands)					
Accounts payable	\$	1,405	\$	1,752		
Accrued compensation and benefits		814		834		
Accrued expenses		2,297		2,686		
	\$	4,516	\$	5,272		

## **10. INVESTMENT TAX CREDITS**

The Company recorded \$253,000 and \$482,000 related to refundable investment tax credits as a reduction of research and development expenses for the three-month period and six-month period ended June 30, 2013, respectively, and \$91,000 and \$1.4 million for the three-month period and six-month period ended June 30, 2012, respectively.

## **11. NET LOSS PER SHARE**

## **Basic and diluted**

Net loss per share is calculated by dividing the net loss of the Company by the weighted average number of shares of common stock outstanding during the year.

	THREE MC ENDE JUNE 3	D	SIX MONTHS ENDED JUNE 30,			
	2013	2012	2013	2012		
Net loss and comprehensive loss for the period (in						
thousands)	\$ (8,012) \$	(4,677)	\$ (12,229) \$	(8,033)		
Weighted average number of common shares	9,957,725	6,358,266	9,957,725	6,358,266		

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Basic and diluted net loss per share	\$	(0.81) \$	(0.73) \$	(1.23) \$	(1.26)		
F-38							

## Mirati Therapeutics, Inc.

#### Notes to Consolidated Financial Statements (Continued)

June 30, 2013 (unaudited)

#### 11. NET LOSS PER SHARE (Continued)

Common stock equivalents from warrants and options were excluded from weighted average number of shares of common stock outstanding for the purpose of calculating diluted net loss per share, because the effect is anti-dilutive.

#### 12. FAIR VALUE MEASUREMENT AND FINANCIAL INSTRUMENTS

The following tables present information about the Company's assets and liabilities that are measured at fair value on a recurring basis for the periods presented and indicates the fair value hierarchy of the valuation techniques we utilized to determine such fair value.

In general, fair values determined by Level 1 inputs utilize quoted prices (unadjusted) in active markets for identical assets or liabilities.

Fair values determined by Level 2 inputs utilize data points that are observable such as quoted prices, interest rates and yield curves.

Fair values determined by Level 3 inputs are unobservable data points for the asset or liability, and include situations where there is little, if any, market activity for the asset or liability.

There were no transfers in or out of Level 1, Level 2 or Level 3 measurements for the periods presented (in thousands):

	JUNE 30,	LEVEL				
	2013	1	LE	VEL 2	LE	VEL 3
Assets						
Cash equivalents	\$ 7,40	)7	\$	7,407	\$	
Marketable securities	10,51	.6		10,516		
Restricted cash equivalents and marketable securities	30	i3		363		
	\$ 18,28	6\$	\$	18,286	\$	
				ĺ		
Liability						
Warrant liability	12,20	8				12,208
	\$ 12,20	8 \$	\$		\$	12,208
	F-39					
		,				

## Mirati Therapeutics, Inc.

## Notes to Consolidated Financial Statements (Continued)

### June 30, 2013 (unaudited)

## 12. FAIR VALUE MEASUREMENT AND FINANCIAL INSTRUMENTS (Continued)

	DECEMB	ER			
	31,	LEVEL	LE	VEL	LEVEL
	2012	1		2	3
Assets					
Cash equivalents	\$ 15	,580 \$	\$	15,580	\$
Marketable securities	18	,580 \$		18,580	
Restricted cash equivalents and marketable securities		374		374	
	\$ 34	,534 \$	\$	34,534	\$
Liability					
Warrant liability					
	\$	\$	\$		\$

The following table presents a reconciliation of the warrant liability measured at fair value using significant unobservable inputs (Level 3) from January 1, 2013 to June 30, 2013 (in thousands):

	THREE MONTHS ENDED JUNE 30,		SIX MONTHS ENDED JUNE 30,		DED	
		2013	2012		2013	2012
Liabilities:						
Balance at beginning of period:	\$	11,823	\$	\$	16,195	\$
Change in fair value of warrant liability included in other (expense)/income		385			(3,987)	
Balance at end of period:	\$	12,208	\$	\$	12,208	\$

The fair value of the warrant liability was calculated utilizing the Black-Scholes option-pricing model, using the following assumptions:

	<b>JANUARY 1, 2013</b>		JUNE 30	, 2013	
	2011	2012	2011	2012	
	Warrants	Warrants	Warrants	Warrants	
Risk-free interest rate	1.2%	1.4%	1.2%	1.4%	
Volatility	107.5%	116.3%	126.4%	116.6%	
Dividend Yield	0%	0%	0%	0%	
Expected life in years	3.3	4.9	2.8	4.4	

## Other financial assets

The Company's other financial assets consist of interest receivable, other receivables and security deposits. The carrying amount of these financial assets is a reasonable approximation of their fair value due to the short-term nature of these financial assets.

## Mirati Therapeutics, Inc.

#### Notes to Consolidated Financial Statements (Continued)

June 30, 2013 (unaudited)

#### 12. FAIR VALUE MEASUREMENT AND FINANCIAL INSTRUMENTS (Continued)

#### Other financial liabilities

The Company's other financial liabilities include accounts payable and accrued liabilities. The carrying value of the accounts payable and accrued liabilities approximates their fair value due to the short-term nature of these financial liabilities.

## **13. CONCENTRATION OF CREDIT RISK**

The maximum exposure to credit risk of the Company at June 30, 2013 is the carrying value of its cash and cash equivalents, marketable securities, restricted cash equivalents and marketable securities, interest receivable, other receivables and security deposits. The Company has an investment policy that monitors the safety and preservation of investments made, which requires them to be highly rated and which limits the amount invested in any one issuer. The investments are reviewed regularly by the Audit Committee.

The Company also manages credit risk by maintaining bank accounts with reputable banks and financial institutions and investing only in investments from banking, governmental or highly rated companies with securities that are traded on active markets and are capable of immediate liquidation, subject to some minor market price variations upon sale.

Cash and cash equivalents and restricted cash were comprised of bankers' acceptances, treasury bills, promissory notes, commercial papers, and term deposits at June 30, 2013 and at December 31, 2012. Cash and cash equivalents and restricted cash as of June 30, 2013 and December 31, 2012 were held in two Canadian chartered banks and one United States bank and follows (in thousands):

				ECEMBER
	JUNE 30, 2013			31,
			2012	
Cash and cash equivalents	\$	9,740	\$	18,403
Restricted cash		363		374
	\$	10,103	\$	18,777

Management has determined that other receivables are collectible and has not recorded a provision against these amounts.

The Company continues to have ongoing license and collaboration agreements with three partners. As per the term of these agreements there was no revenue or expenses with these partners for the period ended June 30, 2013 and 2012.

## **14. SUBSEQUENT EVENTS**

For its financial statements as of June 30, 2013 and for the six-months then ended, the Company evaluated subsequent events through August 9, 2013, the date on which those financial statements were available to be issued.

On July 15, 2013, the Company's common stock was listed on the NASDAQ Capital Market and began trading under the ticker symbol "MRTX". Effective July 26, 2013, the Company voluntarily delisted its shares of common stock from the Toronto Stock Exchange ("TSX").

# 3,250,000 Shares

## **Common Stock**

## PROSPECTUS

Joint Book-Running Managers

# Jefferies Leerink Swann

Lead Manager

# **Piper Jaffray**

October 23, 2013