

**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION**  
Washington, DC 20549

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**FORM 10-Q**

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(Mark One)

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

For the quarterly period ended March 31, 2015

OR

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

Commission file number 001-37359

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**BLUEPRINT MEDICINES CORPORATION**

(Exact Name of Registrant as Specified in Its Charter)

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**Delaware**  
(State or Other Jurisdiction of  
Incorporation or Organization)

**215 First St., Cambridge, Massachusetts**  
(Address of Principal Executive Offices)

**26 3632015**  
(I.R.S. Employer  
Identification No.)

**02142**  
(Zip Code)

**(617) 374-7580**

(Registrant's Telephone Number, Including Area Code)

(Former Name, Former Address and Former Fiscal Year, if Changed Since Last Report)

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

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes  No

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

Large accelerated filer	<input type="checkbox"/>	Accelerated filer	<input type="checkbox"/>
Non-accelerated filer	<input checked="" type="checkbox"/> (Do not check if a smaller reporting company)	Smaller reporting company	<input type="checkbox"/>

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

Number of shares of the registrant's Common Stock, \$0.001 par value, outstanding on June 10, 2015: 27,111,402

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**Blueprint Medicines Corporation**  
**Condensed Balance Sheets**  
(in thousands, except share and per share data)  
*(Unaudited)*

	<u>March 31, 2015</u>	<u>December 31, 2014</u>
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 50,298	\$ 47,240
Restricted cash	119	119
Unbilled accounts receivable	484	—
Prepaid expenses and other current assets	1,279	915
<b>Total current assets</b>	<b>52,180</b>	<b>48,274</b>
Property and equipment, net	1,295	1,482
Other assets	1,099	99
Restricted cash	1,336	70
<b>Total assets</b>	<b>\$ 55,910</b>	<b>\$ 49,925</b>
Liabilities, convertible preferred stock and stockholders' (deficit) equity		
Current liabilities:		
Accounts payable	2,334	814
Accrued expenses	4,404	3,810
Deferred rent	98	138
Restricted stock liability	292	298
Current portion of deferred revenue	5,375	—
Current portion of term loan payable	2,127	1,704
<b>Total current liabilities</b>	<b>14,630</b>	<b>6,764</b>
Restricted stock liability, net of current portion	25	29
Warrant liability	403	365
Deferred revenue, net of current portion	9,457	—
Term loan payable, net of current portion	6,525	7,338
Other long term liabilities	147	—
Commitments		
Series A convertible preferred stock, \$0.001 par value; 40,150,000 shares authorized; 40,000,000 shares issued and outstanding at March 31, 2015 and December 31, 2014 (liquidation preference of \$49,027 at March 31, 2015)	39,958	39,958
Series B convertible preferred stock, \$0.001 par value; 20,999,996 shares authorized; 20,916,663 shares issued and outstanding at March 31, 2015 and December 31, 2014 (liquidation preference of \$27,570 at March 31, 2015)	24,985	24,985
Series C convertible preferred stock, \$0.001 par value; 24,154,589 shares authorized; 24,154,589 shares issued and outstanding at March 31, 2015 and December 31, 2014 (liquidation preference of \$51,578 at March 31, 2015)	49,865	49,868
Stockholders' deficit:		
Common stock, \$0.001 par value; 110,000,000 shares authorized; 2,223,766 and 2,218,652 shares issued at March 31, 2015 and December 31, 2014, respectively, and 1,731,437 and 1,626,738 shares outstanding at March 31, 2015 and December 31, 2014, respectively	2	2
Additional paid-in capital	3,691	2,822
Accumulated deficit	(93,778)	(82,206)
<b>Total stockholders' deficit</b>	<b>(90,085)</b>	<b>(79,382)</b>
<b>Total liabilities, convertible preferred stock, and stockholders' deficit</b>	<b>\$ 55,910</b>	<b>\$ 49,925</b>

**Blueprint Medicines Corporation**  
**Condensed Statements of Operations**  
**(in thousands, except per share data)**  
*(Unaudited)*

	Three Months Ended	
	March 31,	
	2015	2014
Collaboration revenue	\$ 652	\$ —
Operating expenses:		
Research and development	9,232	5,381
General and administrative	2,770	1,572
Total operating expenses	12,002	6,953
Other income (expense):		
Other income (expense), net	(37)	18
Interest expense	(185)	(92)
Total other income (expense)	(222)	(74)
Net loss	<u>\$ (11,572)</u>	<u>\$ (7,027)</u>
Convertible preferred stock dividends	(2,270)	(1,249)
Net loss applicable to common stockholders	<u>\$ (13,842)</u>	<u>\$ (8,276)</u>
Net loss per share applicable to common stockholders — basic and diluted	<u>\$ (8.23)</u>	<u>\$ (6.44)</u>
Weighted-average number of common shares used in net loss per share applicable to common stockholders — basic and diluted	<u>1,681</u>	<u>1,285</u>

**Blueprint Medicines Corporation**  
**Condensed Statements of Cash Flows**  
**(in thousands)**  
**(Unaudited)**

	Three Months Ended	
	March 31,	
	2015	2014
<b>Operating activities</b>		
Net loss	\$ (11,572)	\$ (7,027)
Adjustments to reconcile net loss to net cash used in in operating activities:		
Depreciation and amortization	209	134
Noncash interest expense	29	30
Change in fair value of warrant liability	38	(18)
Stock-based compensation	840	267
Changes in assets and liabilities:		
Unbilled accounts receivable	(484)	—
Prepaid expenses and other current assets	(364)	29
Accounts payable	1,517	(318)
Accrued expenses	(115)	327
Deferred revenue	14,832	—
Deferred rent	(40)	(34)
Net cash provided by (used in) operating activities	4,890	(6,610)
<b>Investing activities</b>		
Purchases of property and equipment	(19)	(94)
Restricted cash	(1,266)	—
Net cash used in investing activities	(1,285)	(94)
<b>Financing activities</b>		
Principal payments on loan payable	(417)	—
Proceeds from issuance of Series B convertible preferred stock, net of issuance costs	—	24,986
Series C convertible preferred stock issuance costs	(3)	—
Payment of offering costs	(146)	—
Proceeds from issuance of common stock	19	—
Net cash provided by (used in) financing activities	(547)	24,986
Net increase in cash and cash equivalents	3,058	18,282
Cash and cash equivalents at beginning of period	47,240	1,987
Cash and cash equivalents at end of period	<u>\$ 50,298</u>	<u>\$ 20,269</u>
<b>Supplemental cash flow information</b>		
Cash paid for interest	<u>\$ 120</u>	<u>\$ 39</u>
Public offering costs incurred but unpaid at period end	<u>\$ 805</u>	<u>\$ —</u>

**Blueprint Medicines Corporation**  
**Notes to Financial Statements**  
*(Unaudited)*

**1. Nature of Business**

Blueprint Medicines Corporation (the Company), a Delaware corporation formed on October 14, 2008, is a biopharmaceutical company focused on improving the lives of patients with genomically defined diseases driven by abnormal kinase activation. The Company's approach is to systematically and reproducibly identify kinases that are drivers of genomically defined diseases and to craft drug candidates with therapeutic windows that provide significant and durable clinical response to patients.

The Company is devoting substantially all of its efforts to research and development, initial market development, and raising capital. The Company is subject to a number of risks similar to those of other early stage companies, including dependence on key individuals; establishing safety and efficacy in clinical trials for its compounds; the need to develop commercially viable drugs; competition from other companies, many of which are larger and better capitalized; and the need to obtain adequate additional financing to fund the development of its drugs. If the Company is unable to raise capital when needed or on attractive terms, it would be forced to delay, reduce, eliminate or out-license certain of its research and development programs or future commercialization efforts.

On May 5, 2015, the Company completed the sale of 9,367,708 shares of its common stock (inclusive of 1,221,874 shares of common stock sold by the Company pursuant to the full exercise of an option to purchase additional shares granted to the underwriters in connection with the offering) in an initial public offering (IPO) at a price to the public of \$18.00 per share, resulting in net proceeds of \$154.2 million after deducting underwriting discounts and commissions and estimated offering costs payable by the Company.

**2. Summary of Significant Accounting Policies**

***Basis of Presentation***

The unaudited interim condensed financial statements of the Company included herein have been prepared in accordance with accounting principles generally accepted in the United States (GAAP) as found in the Accounting Standards Codification (ASC) and Accounting Standards Update (ASU) of the Financial Accounting Standards Board (FASB). Certain information and footnote disclosures normally included in financial statements prepared in accordance with GAAP have been condensed or omitted from this report, as is permitted by such rules and regulations. Accordingly, these financial statements should be read in conjunction with the financial statements as of and for the year ended December 31, 2014 and notes thereto, included in the Company's prospectus filed with the SEC pursuant to Rule 424(b)(4) on April 30, 2015 (the Prospectus).

The unaudited interim condensed financial statements have been prepared on the same basis as the audited financial statements. In the opinion of the Company's management, the accompanying unaudited interim condensed financial statements contain all adjustments which are necessary to present fairly the Company's financial position as of March 31, 2015 and the results of its operations and cash flows for the three months ended March 31, 2015 and 2014. Such adjustments are of a normal and recurring nature. The results for the three months ended March 31, 2015 are not necessarily indicative of the results for the year ending December 31, 2015, or for any future period.

In connection with preparing for its IPO, the Company effected a 1-for-5.5 reverse stock split of the Company's common stock. The reverse stock split became effective on April 10, 2015. All share and per share amounts in the financial statements and notes thereto have been retroactively adjusted for all periods presented to give effect to this reverse stock split, including reclassifying an amount equal to the reduction in par value of common stock to additional paid-in capital. Upon the closing of the IPO, all of the Company's outstanding convertible preferred stock automatically converted into 15,467,479 shares of common stock; and warrants exercisable for convertible preferred stock were automatically converted into warrants exercisable for 42,423 shares of common stock. Additionally, the Company is now authorized to issue 120,000,000 shares of common stock and 5,000,000 shares of preferred stock. The significant

increase in shares outstanding in April 2015 is expected to impact the year-over-year comparability of the Company's net loss per share calculations over the next year.

### ***Use of Estimates***

The preparation of financial statements in conformity with GAAP requires the Company's management to make estimates and assumptions that affect the amounts reported in the financial statements and accompanying notes. Actual results could differ from those estimates. Management considers many factors in selecting appropriate financial accounting policies and in developing the estimates and assumptions that are used in the preparation of the financial statements. Management must apply significant judgment in this process. Management's estimation process often may yield a range of potentially reasonable estimates and management must select an amount that falls within that range of reasonable estimates. Estimates are used in the following areas, among others: stock-based compensation expense, including estimating the fair value of the Company's common stock (the Common Stock); revenue recognition; the valuation of liability-classified warrants; accrued expenses; and income taxes.

### ***Revenue recognition***

The Company recognizes revenue from license and collaboration agreements in accordance with FASB ASC Topic 605, *Revenue Recognition* (ASC 605). Accordingly, revenue is recognized when all of the following criteria are met:

- Persuasive evidence of an arrangement exists;
- Delivery has occurred or services have been rendered;
- The seller's price to the buyer is fixed or determinable; and
- Collectability is reasonably assured.

Amounts received prior to satisfying the revenue recognition criteria are recognized as deferred revenue in the Company's balance sheets. Amounts expected to be recognized as revenue within the 12 months following the balance sheet date are classified as deferred revenue, current portion. Amounts not expected to be recognized as revenue within the 12 months following the balance sheet date are classified as deferred revenue, net of current portion.

The Company's revenue is currently generated through its collaboration agreement with Alexion Pharma Holding (Alexion). The terms of this agreement contains multiple elements, or deliverables, including an exclusive license to research, develop, manufacture and commercialize the licensed products and the compounds in the field in the territory, as well as research and development activities to be performed by the Company on behalf of Alexion related to the licensed product candidates. In addition, the terms of this agreement include payments to the Company of one or more of the following: a nonrefundable, upfront payment; contingent milestone payments related to specified pre-clinical milestones, development milestones and sales-based commercial milestones; fees for research and development services rendered; and royalties on commercial sales of licensed product candidates, if any. To date, the Company has received the upfront payment and is due certain research and development service payments but has not received any milestone payments or earned royalty revenue as a result of product sales. See Note 9 for additional information on this agreement.

When evaluating multiple element arrangements, the Company considers whether the deliverables under the arrangement represent separate units of accounting. This evaluation requires subjective determinations and requires management to make judgments about the individual deliverables and whether such deliverables are separable from the other aspects of the contractual relationship. In determining the units of accounting, management evaluates certain criteria, including whether the deliverables have standalone value, based on the consideration of the relevant facts and circumstances for each arrangement. The consideration received is allocated among the separate units of accounting using the relative selling price method, and the applicable revenue recognition criteria are applied to each of the separate units. Deliverables are considered separate units of accounting provided that: (i) the delivered item(s) has value to the customer on a stand-alone basis and (ii) if the arrangement includes a general right of return relative to the delivered item(s), delivery or performance of the undelivered item(s) is considered probable and substantially in the control of the

Company. In assessing whether an item has stand-alone value, the Company considers factors such as the research, manufacturing and commercialization capabilities of the collaboration partner and the availability of the associated expertise in the general marketplace. In addition, the Company considers whether the collaboration partner can use the deliverable(s) for their intended purpose without the receipt of the remaining element(s), whether the value of the deliverable is dependent on the undelivered item(s) and whether there are other vendors that can provide the undelivered element(s). The Company's collaboration agreement with Alexion does not contain a general right of return relative to the delivered item(s).

Arrangement consideration that is fixed or determinable is allocated among the separate units of accounting using the relative selling price method. Then, the applicable revenue recognition criteria in ASC 605-25 are applied to each of the separate units of accounting in determining the appropriate period and pattern of recognition. The Company determines the selling price of a unit of accounting following the hierarchy of evidence prescribed by ASC 605-25. Accordingly, the Company determines the estimated selling price for units of accounting within each arrangement using vendor-specific objective evidence (VSOE) of selling price, if available, third-party evidence (TPE) of selling price if VSOE is not available, or best estimate of selling price (BESP) if neither VSOE nor TPE is available. The Company typically uses BESP to estimate the selling price, since it generally does not have VSOE or TPE of selling price for its units of accounting. Determining the BESP for a unit of accounting requires significant judgment. In developing the BESP for a unit of accounting, the Company considers applicable market conditions and relevant entity-specific factors, including factors that were contemplated in negotiating the agreement with the customer and estimated costs. The Company validates the BESP for units of accounting by evaluating whether changes in the key assumptions used to determine the BESP will have a significant effect on the allocation of arrangement consideration between multiple units of accounting.

In the event that an element of a multiple element arrangement does not represent a separate unit of accounting, the Company recognizes revenue from the combined element over the period over which it expects to fulfill its performance obligations or as undelivered items are delivered, as appropriate, if all of the other revenue recognition criteria in ASC 605-25 are met. If the pattern of performance in which the service is provided to the customer can be determined and objectively measurable performance measures exist, then the Company recognizes revenue under the arrangement using the proportional performance method. If there is no discernible pattern of performance and/or objectively measurable performance measures do not exist, then the Company recognizes revenue under the arrangement on a straight-line basis over the period the Company is expected to complete its performance obligations. Revenue recognized is limited to the lesser of the cumulative amount of payments received or the cumulative amount of revenue earned, as determined using the straight-line method or proportional performance method, as applicable, as of the period ending date.

The Company's multiple-element revenue arrangements may include the following

*Exclusive Licenses*

The deliverables under the Company's collaboration agreements may include exclusive licenses to research, develop, manufacture and commercialize licensed products. To account for this element of an arrangement, management evaluates whether an exclusive license has stand-alone value from the undelivered elements based on the consideration of the relevant facts and circumstances of the arrangement, including the research and development capabilities of the collaboration partner. The Company may recognize the arrangement consideration allocated to licenses upon delivery of the license if facts and circumstances indicate that the license has stand-alone value from the undelivered elements, which generally include research and development services. The Company defers arrangement consideration allocated to licenses if facts and circumstances indicate that the delivered license does not have stand-alone value from the undelivered elements.

When management believes a license does not have stand-alone value from the other deliverables to be provided in the arrangement, the Company recognizes revenue attributed to the license on a proportional basis over the Company's contractual or estimated performance period, which is typically the term of the Company's research and development obligations. If management cannot reasonably estimate when the Company's performance obligation ends, then revenue is deferred until management can reasonably estimate when the performance obligation ends. The periods



over which revenue should be recognized are subject to estimates by management and may change over the course of the research and development and licensing agreement. Such a change could have a material impact on the amount of revenue the Company records in future periods.

#### *Research and Development Services*

The deliverables under the Company's collaboration agreements may include research and development services to be performed by the Company on behalf of the partner. Payments or reimbursements resulting from the Company's research and development efforts are recognized as the services are performed and presented on a gross basis because the Company is the principal for such efforts, so long as there is persuasive evidence of an arrangement, the fee is fixed or determinable, and collection of the related amount is reasonably assured.

#### *Milestone Revenue*

The Company's collaboration agreements may include contingent milestone payments related to specified pre-clinical milestones, development milestones and sales-based commercial milestones.

At the inception of an arrangement that includes milestone payments, the Company evaluates whether each milestone is substantive and at risk to both parties on the basis of the contingent nature of the milestone. This evaluation includes an assessment of whether:

- the consideration is commensurate with either the Company's performance to achieve the milestone or the enhancement of the value of the delivered item(s) as a result of a specific outcome resulting from the Company's performance to achieve the milestone;
- the consideration relates solely to past performance; and
- the consideration is reasonable relative to all of the deliverables and payment terms within the arrangement.

The Company evaluates factors such as the scientific, clinical, regulatory, commercial and other risks that must be overcome to achieve the respective milestone and the level of effort and investment required to achieve the respective milestone in making this assessment. There is considerable judgment involved in determining whether a milestone satisfies all of the criteria required to conclude that a milestone is substantive. Milestones that are not considered substantive are accounted for as license payments and recognized over the remaining period of performance from the date of achievement of the milestone. Milestones that are considered substantive will be recognized in their entirety upon successful accomplishment of the milestone, assuming all other revenue recognition criteria are met.

#### *Royalty Revenue*

The Company will recognize royalty revenue in the period of sale of the related product(s), based on the underlying contract terms, provided that the reported sales are reliably measurable and the Company has no remaining performance obligations, assuming all other revenue recognition criteria are met.

#### **Cash Equivalents**

Cash equivalents are highly liquid investments that are readily convertible into cash with original maturities of three months or less when purchased. These assets include an investment in a money market fund that invests in U.S. Treasury obligations. Cash equivalents consist of the following at March 31, 2015 and December 31, 2014 (in thousands):

	March 31, 2015	December 31, 2014
Money market fund	\$ 50,298	\$ 47,240

**Fair Value of Financial Instruments**

The fair value hierarchy prioritizes the inputs to valuation techniques used to measure fair value into three broad levels as follows:

Level 1 inputs are quoted prices (unadjusted) in active markets for identical assets or liabilities.

Level 2 inputs are inputs other than quoted prices included within Level 1 that are observable for the asset or liability, either directly or indirectly.

Level 3 inputs are unobservable inputs that reflect the Company's own assumptions about the assumptions market participants would use in pricing the asset or liability. Financial assets and liabilities are classified in their entirety based on the lowest level of input that is significant to the fair value measurement.

Financial instruments measured at fair value as of March 31, 2015, are classified below based on the fair value hierarchy described above:

Description	March 31, 2015	Active Markets (Level 1)	Observable Inputs (Level 2)	Unobservable Inputs (Level 3)
Money market funds, included in cash equivalents	\$ 50,298	\$ 50,298	\$ —	\$ —
Preferred stock warrants	(403)	—	—	(403)

Financial instruments measured at fair value as of December 31, 2014, are classified below based on the fair value hierarchy described above:

Description	December 31, 2014	Active Markets (Level 1)	Observable Inputs (Level 2)	Unobservable Inputs (Level 3)
Money market funds, included in cash equivalents	\$ 47,240	\$ 47,240	\$ —	\$ —
Preferred stock warrants	(365)	—	—	(365)

At March 31, 2015 and December 31, 2014, all of the Company's cash equivalents comprise of a money market account, the fair value of which is valued using Level 1 inputs. The fair value of the Company's term loan payable is determined using current applicable rates for similar instruments as of the balance sheet date. The carrying value of the Company's term loan payable approximates fair value because the Company's interest rate yield approximates current market rates. The Company's term loan payable is a Level 3 liability within the fair value hierarchy. The fair value of the preferred stock warrant liability was determined based on Level 3 inputs and utilizing the Black- Scholes option pricing model (Note 5).

**Deferred Offering Costs**

The Company capitalizes certain legal, accounting and other third-party fees that are directly associated with in-process equity financings as other assets until such financings are consummated. After consummation of the equity financing, these costs are recorded in stockholders' equity (deficit) as a reduction of additional paid-in capital generated as a result of the offering. As of March 31, 2015 and December 31, 2014, the Company recorded \$1,093 and \$91, respectively, of deferred offering costs, included in other assets in the accompanying balance sheet, in contemplation of its IPO.

There have been no other material changes to the significant accounting policies previously disclosed in the Company's Prospectus.

### **Recent Accounting Pronouncements**

In May 2014, the FASB issued ASU No. 2014-09, Revenue from Contracts with Customers (ASU 2014-09), which supersedes the revenue recognition requirements in ASC 605 and most industry-specific guidance. The new standard requires that an entity recognize revenue to depict the transfer of promised goods or services to customers in an amount that reflects the consideration to which the company expects to be entitled in exchange for those goods or services. The update also requires additional disclosure about the nature, amount, timing and uncertainty of revenue and cash flows arising from customer contracts, including significant judgments and changes in judgments and assets recognized from costs incurred to obtain or fulfill a contract. This new guidance is expected to be effective for annual reporting periods (including interim reporting periods within those years) beginning January 1, 2018; early adoption in 2017 is permitted. Companies have the option of applying this new guidance retrospectively to each prior reporting period presented or retrospectively with the cumulative effect of initially applying this update recognized at the date of initial application. The Company has not yet determined the potential effects of the adoption of this standard on its consolidated financial position, results of operations or cash flows.

On April 7, 2015, the FASB, issued ASU 2015-03, *Simplifying the Presentation of Debt Issuance Costs* (ASU 2015-03), which requires debt issuance costs to be presented in the balance sheet as a direct deduction from the carrying value of the associated debt liability, consistent with the presentation of a debt discount. ASU 2015-03 will be effective for the Company on January 1, 2016, with early adoption permitted. ASU 2015-03 will be applied on a retrospective basis. The Company is currently assessing the potential impact of the adoption of ASU 2015-03 on its financial statements.

In 2014, the FASB issued new guidance on management's responsibility in evaluating whether or not there is substantial doubt about a company's ability to continue as a going concern within one year from the date the financial statements are issued each reporting period. This new accounting guidance is effective for annual periods ending after December 15, 2016. Early adoption is permitted. The Company is in process of evaluating the new guidance and determining the expected effect on its financial statements.

### **3. Restricted Cash**

At March 31, 2015 and December 31, 2014, \$1.5 million and \$0.2 million, respectively, of the Company's cash is restricted by a bank. As of March 31, 2015, \$0.1 million of the restricted cash is included in current assets as collateral for a stand-by letter of credit issued by the Company to its landlord in connection with the lease of the Company's corporate headquarters. On February 12, 2015, the Company entered into a lease for approximately 38,500 rentable square feet of office and laboratory space in Cambridge, Massachusetts, with a lease term commencing June 15, 2015 and ending on October 31, 2022, assuming occupancy in October 2015. The lease agreement required the Company to pay a security deposit of \$1.3 million, which is included in long-term assets on the Company's balance sheet as of March 31, 2015. In addition, \$0.1 million of restricted cash is included in long-term assets related to the Company's corporate credit card agreement as of March 31, 2015 and December 31, 2014.

#### 4. Accrued Expenses

Accrued expenses consist of the following (in thousands):

	<u>March 31,</u> <u>2015</u>	<u>December 31,</u> <u>2014</u>
Employee compensation	\$ 625	\$ 623
External research and pre-clinical development	1,597	2,034
Severance	541	330
Consulting	288	216
Interest	40	150
Deferred offering costs	805	71
Other	508	386
	<u>\$ 4,404</u>	<u>\$ 3,810</u>

#### 5. Term Loan

In May 2013, the Company entered into a loan and security agreement with Silicon Valley Bank (the 2013 Term Loan), which provided for up to \$5.0 million in funding, to be made available in three tranches. Loan advances accrue interest at a fixed rate of 2% above the Prime Rate. In June 2013, the Company drew the first loan advance of \$1.0 million under the 2013 Term Loan and was required to make interest-only payments until April 1, 2014, and consecutive monthly payments of principal, plus accrued interest, over the remaining term through March 2017. In September 2013, the Company drew the second loan advance of \$2.0 million under the 2013 Term Loan and was required to make interest-only payments until April 1, 2014, and consecutive monthly payments of principal, plus accrued interest, over the remaining term through March 2017. In June 2014, the Company drew the remaining \$2.0 million advance under the 2013 Term Loan and is required to make interest-only payments until January 1, 2015, and consecutive monthly payments of principal, plus accrued interest, over the remaining term through December 2017. In November 2014, the Company amended the 2013 Term Loan to allow the Company to borrow an additional \$5.0 million (the 2014 Term Loan). The Company accounted for the amendment as a modification to the existing 2013 Term Loan. The Company immediately drew the additional \$5.0 million under the 2014 Term Loan and is required to make interest-only payments until December 1, 2015, and consecutive monthly payments of principal, plus accrued interest, over the remaining term through November 2018. The Company is required to pay a fee of 4% of the total loan advances at the end of the term of each of the 2013 Term Loan and the 2014 Term Loan. The fee is being accreted to interest expense over the term of the 2013 Term Loan and the 2014 Term Loan. In the event of prepayment, the Company is obligated to pay 1% to 2% of the amount of the outstanding principal depending upon the timing of the prepayment.

The 2013 Term Loan and 2014 Term Loan are collateralized by a blanket lien on all corporate assets, excluding intellectual property, and by a negative pledge of the Company's intellectual property. The 2013 Term Loan and 2014 Term Loan contain customary default provisions that include material adverse events, as defined therein. The Company has determined that the risk of subjective acceleration under the material adverse events clause is remote and therefore has classified the outstanding principal in current and long-term liabilities based on scheduled principal payments.

The Company assessed all terms and features of the 2013 Term Loan and the 2014 Term Loan in order to identify any potential embedded features that would require bifurcation. As part of this analysis, the Company assessed the economic characteristics and risks of the term loan, including put and call features. The Company determined that all features of each of the 2013 Term Loan and the 2014 Term Loan are clearly and closely associated with a debt host and do not require bifurcation as a derivative liability, or the fair value of the feature is immaterial to the Company's financial statements. The Company will continue to reassess the features on a quarterly basis to determine if they require separate accounting.

Future minimum payments, which include principal and interest due under each of the 2013 Term Loan and the 2014 Term Loan, are \$1.7 million, in the aggregate, for the remainder of 2015.

In connection with the 2013 Term Loan, the Company issued a warrant to Silicon Valley Bank to purchase 150,000 shares of Series A Convertible Preferred Stock at an exercise price of \$1.00 per share (the Series A Warrant). In connection with the 2014 Term Loan, the Company issued an additional warrant to Silicon Valley Bank to purchase 83,333 shares of Series B Convertible Preferred Stock at an exercise price of \$1.20 per share (the Series B Warrant). Both warrants were exercisable immediately and have a ten-year life. No portion of the warrants have been exercised as of March 31, 2015.

The Series A Warrant and the Series B Warrant are classified as a liability and are re-measured to the then current fair value at each balance sheet date. Re-measurement gains or losses are recorded in other income (expense) in the statements of operations. The following table sets forth a summary of changes in the fair value of the warrants which represents a recurring measurement that is classified within Level 3 of the fair value hierarchy wherein fair value is estimated using significant unobservable inputs (in thousands):

	Year Ended	
	Three Months Ended March 31,	
	2015	2014
Beginning balance	\$ 365	\$ 119
Change in fair value	38	(18)
Ending balance	\$ 403	\$ 101

The Company initially valued the Series A Warrant and the Series B Warrant at issuance and at the balance sheet dates using the Black-Scholes option pricing model. The significant assumptions used in estimating the fair value of the warrants include the volatility of the stock underlying the warrant, risk-free interest rate, estimated fair value of the preferred stock underlying the warrant, and the estimated term of the warrant. The fair value of the preferred stock underlying the warrants was estimated using the implied value from the common stock valuations on those dates. The Company used the following weighted-average assumptions in its Black-Scholes option pricing model:

	Series A Warrant			Series B Warrant	
	Issuance	March 31, 2015	March 31, 2014	Issuance	March 31, 2015
Fair value of underlying instrument	\$ 1.00	\$ 1.93	\$ 0.83	\$ 1.97	\$ 2.03
Expected volatility	80.70 %	91.70 %	86.49 %	87.18 %	88.00 %
Expected term (in years)	10.0	8.15	9.2	10.0	9.6
Risk-free interest rate	2.58 %	1.83 %	2.72 %	2.36 %	1.94 %
Expected dividend yield	— %	— %	— %	— %	— %

The Company recorded a debt discount upon issuance of the warrants, which is being accreted as interest expense over the remaining term of the loan. The Company also recorded a warrant liability that is classified as a long-term liability in the accompanying balance sheets. The Company recorded interest expense related to the Series A Warrant and the Series B Warrant of less than \$0.1 million in each of the three months ended March 31, 2015 and 2014.

Upon completion of the IPO, the Series A Warrant became exercisable for 27,272 shares of the Common Stock at an exercise price of \$5.50 per share, and the Series B Warrant became exercisable for 15,151 shares of the Common Stock at an exercise price of \$6.60 per share.

On May 13, 2015, Silicon Valley Bank exercised the Series A Warrant and the Series B Warrant pursuant to the cashless exercise feature of the warrants. In connection with the exercise of the Series A Warrant under the 2013 Term Loan, the Company issued 21,281 shares of Common Stock. Warrants to purchase 5,991 shares of Common Stock were cancelled as payment for the aggregate exercise price of the Series A Warrant. In connection with the exercise of the Series B Warrant under the 2014 Term Loan, the Company issued 11,157 shares of Common Stock. Warrants to purchase 3,994 shares of Common Stock were cancelled as payment for the aggregate exercise price of the Series B Warrant.

## 6. Stock Awards

The Company grants restricted stock awards, incentive stock options (ISO), and nonstatutory stock options (NSO) under the Blueprint Medicines Corporation 2011 Stock Option and Grant Plan (the Plan), as amended and restated. In February 2015, the Company amended the Blueprint Medicines Corporation 2011 Stock Option and Grant Plan to increase the number of shares available for grant to 3,590,927. At March 31, 2015, there were 190,276 shares available for future grant under the Plan. ISOs may not be granted at less than fair value on the date of the grant. Furthermore, the exercise price of ISOs granted to an employee, who at the time of grant is a 10% shareholder, may not be less than 110% of the fair value on the date of grant.

Terms of restricted stock awards and stock option agreements, including vesting requirements, are determined by the board of directors, subject to the provisions of the Plan. Options and restricted stock awards granted by the Company generally vest ratably over four years, with a one - year cliff for new employee awards, and are exercisable from the date of grant for a period of ten years. For options and restricted stock awards granted to date, the exercise price equaled the estimated fair value of the Common Stock as determined by the board of directors on the date of grant. The dates of the Company's contemporaneous valuations have not always coincided with the dates of the stock option grants. For financial reporting purposes, the Company performed common stock valuations with the assistance of a third-party specialist, as of January 6, 2014, July 30, 2014, November 10, 2014, February 1, 2015 and March 1, 2015 to determine stock-based compensation expense.

A summary of the Company's unvested restricted stock and related information follows:

	Shares	Weighted-Average Grant Date Fair Value
Unvested at December 31, 2014	425,279	\$ 0.46
Granted	—	—
Vested	(82,045)	0.30
Repurchased	(15,434)	0.55
Unvested at March 31, 2015	<u>327,800</u>	0.49

The Company has granted restricted stock to non-employees which contain both performance-based and service-based vesting criteria. Stock-based compensation expense associated with these performance-based awards is recognized if the performance condition is considered probable of achievement using management's best estimates. In the three months ended March 31, 2014 management concluded that the milestones associated with 90,909 shares of performance-based restricted stock were probable of achievement, and the Company began to record stock-based compensation expense using the accelerated attribution method, accordingly. The Company recorded \$0.1 million of stock-based compensation expense for non-employee performance-based awards in the three months end March 31, 2014. In the three months ended December 31, 2014, management concluded that the milestones associated with an additional 90,909 shares of performance-based restricted stock were probable of achievement, and the Company began to record stock-based compensation expense using the accelerated attribution method accordingly. The Company recorded \$0.2 million of stock-based compensation expense for non-employee performance-based awards in the three months end March 31, 2015 related to both milestone achievements.

A summary of the Company's stock option activity and related information follows:

	Shares	Weighted-Average Exercise Price	Remaining Contractual Life (in Years)	Aggregate Intrinsic Value(3) (in thousands)
Outstanding at December 31, 2014(1)	1,501,912	\$ 2.02	9.42	\$ 7,704
Granted	572,638	8.92		
Exercised	(12,652)	1.81		
Canceled	(10,270)	2.47		
Outstanding at March 31, 2015(1)	2,051,628	\$ 3.95	9.37	\$ 11,312
Exercisable at March 31, 2015	241,376	\$ 2.06	8.97	\$ 1,787
Vested and expected to vest at March 31, 2015(2)	1,949,469	\$ 3.94	9.37	\$ 10,760

(1) Includes 164,529 unvested shares of common stock related to early exercises of stock options.

(2) Represents the number of vested options as of March 31, 2015, plus the number of unvested options expected to vest as of March 31, 2015.

(3) Intrinsic value represents the amount by which the fair market value as of March 31, 2015 of the underlying common stock exceeds the exercise price of the option.

The fair value of stock options is estimated on the grant date using the Black-Scholes option-pricing model based on the following weighted average assumptions:

	Three Months Ended	
	March 31, 2015	March 31, 2014
Risk-free interest rate	1.41% - 1.67 %	1.88 %
Expected dividend yield	— %	— %
Expected term (years)	6	6
Expected stock price volatility	86.14 %	91.36 %

The weighted-average grant date fair value of options granted in the three months ended March 31, 2015 and 2014 was \$6.45 and \$1.41, respectively. The total intrinsic value of options exercised in the three months ended March 31, 2015 was \$0.1 million. There were no options exercised in the three months ended March 31, 2014.

Total stock-based compensation expense recognized for all stock-based compensation awards in the statements of operations is as follows (in thousands):

	Three Months Ended	
	March 31, 2015	March 31, 2014
Research and development	\$ 392	\$ 131
General and administrative	448	136
Total stock-based compensation expense	\$ 840	\$ 267

At March 31, 2015, there was \$7.5 million of total unrecognized compensation cost related to nonvested stock awards, which is expected to be recognized over a weighted-average period of 3.17 years. Due to an operating loss, the Company does not record tax benefits associated with stock-based compensation or option exercises. Tax benefit will be recorded when realized.

## 7. Net Loss per Share Applicable to Common Stockholders

Basic net loss per share applicable to common stockholders is calculated by dividing net loss applicable to common stockholders by the weighted average shares outstanding during the period, without consideration for common stock equivalents. Net loss applicable to common stockholders is calculated by adjusting the net loss of the Company for cumulative preferred stock dividends. Diluted net loss per share applicable to common stockholders is calculated by adjusting weighted average shares outstanding for the dilutive effect of common stock equivalents outstanding for the period. For purposes of the dilutive net loss per share applicable to common stockholders calculation, convertible preferred stock, warrants, stock options, and unvested restricted stock are considered to be common stock equivalents but are excluded from the calculation of diluted net loss per share applicable to common stockholders, as their effect would be anti-dilutive; therefore, basic and diluted net loss per share applicable to common stockholders were the same for all periods presented. The following common stock equivalents were excluded from the calculation of diluted net loss per share applicable to common stockholders for the periods indicated because including them would have had an anti-dilutive effect.

	Three Months Ended	
	March 31,	
	2015	2014
Convertible preferred stock	15,467,479	11,075,750
Warrants	42,423	27,272
Stock options	2,051,628	482,156
Unvested restricted stock	327,800	720,276
Total	17,889,330	12,305,454

The weighted average number of common shares used in net loss per share applicable to common stockholders on a basic and diluted basis were 1,681,236 and 1,285,168 for the three months ended March 31, 2015 and 2014, respectively.

## 8. Collaboration Agreement

### *Alexion*

In March 2015, the Company entered into a research, development and commercialization agreement with Alexion to research, develop and commercialize drug candidates for an undisclosed activated kinase target, which is the cause of a rare genetic disease. Under the terms of this agreement, the Company is responsible for research and pre-clinical development activities related to drug candidates and Alexion is responsible for all clinical development, manufacturing and commercialization activities related to drug candidates.

Alexion is responsible for funding 100% of the Company's research and development costs incurred under the research plan, including pass-through costs and its employees' time devoted to the research plan at a negotiated yearly rate per full-time equivalent for its employees' time and their associated overhead expenses. The Company received a \$15.0 million non-refundable upfront payment in March 2015 upon execution of the agreement and is eligible to receive over \$250 million in payments upon the successful achievement of pre-specified pre-clinical, clinical, regulatory and commercial milestones as follows: (i) up to \$6.0 million in pre-clinical milestone payments for the first licensed product, (ii) up to \$83.0 million and \$61.5 million in development milestone payments for the first and second licensed products, respectively, and (iii) up to \$51.0 million in commercial milestone payments for each of the first and second licensed products. Alexion will pay the Company tiered royalties, ranging from mid-single to low-double digit percentages royalties, on a country-by-country and licensed-product-by-licensed-product basis, on worldwide net product sales of licensed products. The royalty term for each licensed product in each country is the period commencing with first commercial sale of such licensed product in such country and ending on the later of (i) the expiration of the last-to-expire



valid claim of specified patents covering such licensed product, (ii) the expiration of the applicable regulatory exclusivity period, and (iii) 10 or 15 years from specified commercial sales. There are no refund provisions in the agreement.

Alexion has the right to terminate the Alexion agreement if the Company undergoes a change of control or become an affiliate of a biotechnology or pharmaceutical company, and may terminate at-will upon 90 days, prior written notice. The Company and Alexion have the right to terminate the agreement in the event of the other party's uncured breach or insolvency, and in certain other circumstances agreed to by the parties

The Company determined that there were three deliverables under the agreement: (i) an exclusive license to research, develop, manufacture and commercialize the licensed products and the compounds in the field in the territory, (ii) conducting research and development activities under the research plan and (iii) participation on a joint steering committee (JSC) and joint project team (JPT).

The Company determined that the license did not have value to Alexion on a stand-alone basis due to the specialized nature of the research services to be provided by the Company that are not available in the marketplace. Therefore, the deliverables are not separable and, accordingly, the license, undelivered research and development activities and JSC and JPT participation are a single unit of accounting. When multiple deliverables are accounted for as a single unit of accounting, the Company bases its revenue recognition model on the final deliverable. Under the agreement, the last deliverable to be completed is its research and development activities and participation on the JSC and JPT, which are expected to be delivered over the same performance period. The Company is utilizing a proportional performance model to recognize revenue under the agreement. During the three months ended March 31, 2015, the Company recognized revenue of \$0.7 million related to the agreement.

The Company evaluated whether the milestones that may be received in connection with the agreement are substantive or non-substantive milestones.

The Company concluded that the first pre-clinical milestone in the agreement is non-substantive due to the certainty at the date the arrangement was entered into that the event will be achieved. The Company will recognize revenues from the related milestone payment over the period of performance. During May 2015, the Company achieved the first pre-clinical milestone of \$1.75 million under the terms of the agreement.

The remaining non-refundable pre-clinical milestones that are expected to be achieved as a result of the Company's efforts during the period of substantial involvement are considered substantive and are recognized as revenue upon the achievement of the milestone, assuming all other revenue recognition criteria are met. Milestones that are not considered substantive because the Company does not contribute effort to the achievement of such milestones are generally achieved after the period of substantial involvement and are recognized as revenue upon achievement of the milestone, as there are no undelivered elements remaining and no continuing performance obligations, assuming all other revenue recognition criteria are met.

## **9. Related-Party Transactions**

The Company has received consulting and management services from one of its investors, Third Rock Ventures LLC (Third Rock Ventures). The Company paid Third Rock Ventures \$0.1 million for these services during the three months ended March 31, 2014. The Company did not receive any consulting services from Third Rock Ventures during the three months ended March 31, 2015.

## **10. Subsequent Events**

### ***2015 Stock Option and Incentive Plan***

On April 8, 2015, the 2015 Stock Option and Incentive Plan was adopted by the board of directors and approved by the stockholders and became effective upon the completion of the IPO. The 2015 Stock Option Plan

replaced the 2011 Stock Option Plan. Any options or awards outstanding under the Company's 2011 Stock Option Plan remained outstanding and effective. The 2015 Stock Option Plan provides the Company flexibility to use various equity-based incentive and other awards as compensation tools to motivate its workforce. These tools include stock options, stock appreciation rights, restricted stock, restricted stock units, unrestricted stock, performance share awards and cash-based awards. The Company initially reserved 1,460,084 shares of common stock for the issuance of awards under the 2015 Stock Option Plan, which will be cumulatively increased on January 1 of each calendar year by 4% of the number of shares of common stock issued and outstanding on the immediately preceding December 31 or such lesser amount as specified by the compensation committee of the board of directors. This number is subject to adjustment in the event of a stock split, stock dividend or other change in our capitalization.

***2015 Employee Stock Purchase Plan***

On April 8, 2015, the Company's stockholders approved the 2015 Employee Stock Purchase Plan. A total of 243,347 shares of common stock were initially authorized for issuance under this plan. The 2015 Employee Stock Purchase Plan became effective upon the completion of the IPO.

## Item 2. Management's Discussion And Analysis Of Financial Condition And Results Of Operations

*The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our unaudited financial statements and related notes appearing elsewhere in this Quarterly Report on Form 10-Q and the audited financial information and the notes thereto included in our final prospectus for our initial public offering filed pursuant to Rule 424(b) under the Securities Act of 1933, as amended, or the Securities Act, with the Securities and Exchange Commission, or the SEC on April 30, 2015, or the Prospectus.*

*Our actual results and timing of certain events may differ materially from the results discussed, projected, anticipated, or indicated in any forward-looking statements. We caution you that forward-looking statements are not guarantees of future performance and that our actual results of operations, financial condition and liquidity, and the development of the industry in which we operate may differ materially from the forward-looking statements contained in this Quarterly Report. In addition, even if our results of operations, financial condition and liquidity, and the development of the industry in which we operate are consistent with the forward-looking statements contained in this Quarterly Report, they may not be predictive of results or developments in future periods.*

*The following information and any forward-looking statements should be considered in light of factors discussed elsewhere in the Quarterly Report on Form 10-Q, including those risks identified under Part II, Item 1A. Risk Factors.*

*We caution readers not to place undue reliance on any forward-looking statements made by us, which speak only as of the date they are made. We disclaim any obligation, except as specifically required by law and the rules of the SEC, to publicly update or revise any such statements to reflect any change in our expectations or in events, conditions or circumstances on which any such statements may be based, or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements.*

### Overview

We are a biopharmaceutical company focused on improving the lives of patients with genomically defined diseases driven by abnormal kinase activation. Our approach is to systematically and reproducibly identify kinases that are drivers of genomically defined diseases and to craft drug candidates with therapeutic windows that provide significant and durable clinical responses to patients. This integrated biology and chemistry approach enables us to drug known kinases that have been difficult to inhibit selectively and also identify, characterize and drug novel kinase targets. By focusing on genomically defined diseases, we believe that we will have a more efficient development path with a greater likelihood of success. Over the past three years, we have developed a robust small molecule drug pipeline in cancer and a rare genetic disease. One of our lead drug candidates is BLU-285, which targets KIT Exon 17 and PDGFR $\alpha$  D842V, abnormally active receptor tyrosine kinase mutants that are drivers of cancer and proliferative disorders. BLU-285 will initially be developed for patients with systemic mastocytosis, a myeloproliferative disorder of the mast cells, and defined subsets of patients with gastrointestinal stromal tumor, the most common sarcoma, or tumor of bone or connective tissue, of the gastrointestinal tract. Our other lead drug candidate is BLU-554, which targets FGFR4, a kinase that is aberrantly activated and is a driver of disease in a defined subset of patients with hepatocellular carcinoma, the most common type of liver cancer. Both drug candidates have demonstrated proof of concept in pre-clinical models and we are on track to file Investigational New Drug applications, or INDs, in mid-2015 and initiate our Phase 1 clinical trials in mid-2015. We are also developing a drug candidate to target both RET, a receptor tyrosine kinase that can become abnormally activated when a portion of the gene that encodes RET is joined to part of another gene, and RET resistant mutants that we predict will arise from treatment with first generation therapies. We believe that our strategy will allow us to deliver transformative drugs to patients while building a fully-integrated biopharmaceutical company.

In March 2015, we entered into a research, development and commercialization agreement, with Alexion Pharma Holding, or Alexion, to research, develop and commercialize drug candidates for an undisclosed activated kinase target, which is the cause of a rare genetic disease. Under the terms of this agreement, we are responsible for research and pre-clinical development activities related to drug candidates and Alexion is responsible for all clinical development, manufacturing and commercialization activities related to drug candidates.

Alexion is responsible for funding 100% of our research and development costs incurred under the research plan, including pass-through costs and our employees' time devoted to the research plan at a negotiated yearly rate per full-time equivalent for our employees' time and their associated overhead expenses. We received a \$15.0 million non-refundable upfront payment in March 2015 upon execution of the agreement and are eligible to receive over \$250 million in payments upon the successful achievement of pre-specified pre-clinical, clinical, regulatory and commercial milestones as follows: (i) up to \$6.0 million in pre-clinical milestone payments for the first licensed product, (ii) up to \$83.0 million and \$61.5 million in development milestone payments for the first and second licensed products, respectively, and (iii) up to \$51.0 million in commercial milestone payments for each of the first and second licensed products. Alexion will pay us tiered royalties, ranging from mid-single to low-double digit percentages royalties, on a country-by-country and licensed- product-by-licensed product basis, on worldwide net product sales of licensed products. The royalty term for each licensed product in each country is the period commencing with first commercial sale of such licensed product in such country and ending on the later of (i) the expiration of the last-to-expire valid claim of specified patents covering such licensed product, (ii) the expiration of the applicable regulatory exclusivity period, and (iii) 10 or 15 years from specified commercial sales.

Alexion has the right to terminate the agreement if we undergo a change of control or become an affiliate of a biotechnology or pharmaceutical company, and may terminate at-will upon 90 days, prior written notice. We and Alexion have the right to terminate the Alexion agreement in the event of the other party's uncured breach or insolvency, and in certain other circumstances agreed to by the parties.

During the three months ended March 31, 2015, we recognized revenue of \$0.7 million related to the Alexion agreement. Since inception, our operations have focused on organizing and staffing our company, business planning, raising capital, establishing our intellectual property, building our platform including our proprietary compound library and new target discovery engine, identifying kinase drug targets and potential drug candidates, producing drug substance and drug product material for use in pre-clinical studies, conducting pre-clinical studies, including Good Laboratory Practice, or GLP, toxicology studies and preparing to commence planned clinical development activities. We expect to begin conducting clinical trials in mid-2015. We do not have any drugs approved for sale and have not generated any revenue from drug sales. From inception through March 31, 2015, we had funded our operations primarily through private placements of our convertible preferred stock and debt financing. We raised an aggregate of \$125.1 million of gross proceeds to fund our operations, of which \$115.1 million was from the issuance of convertible preferred stock and \$10.0 million was from a debt financing. On May 5, 2015, we completed the sale of 9,367,708 shares of our common stock (inclusive of 1,221,874 shares of common stock sold by us pursuant to the full exercise of an option to purchase additional shares granted to the underwriters in connection with the offering) in our initial public offering, or IPO, at a price to the public of \$18.00 per share, resulting in net proceeds of \$154.2 million after deducting underwriting discounts and commissions and estimated offering costs payable by us. The shares began trading on the NASDAQ Global Select Market on April 30, 2015. Upon the closing of the IPO, all outstanding shares of convertible preferred stock converted into 15,467,479 shares of common stock; and warrants exercisable for convertible preferred stock were automatically converted into warrants exercisable for 42,423 shares of common stock. Additionally, we are now authorized to issue 120,000,000 shares of common stock and 5,000,000 shares of preferred stock.

Since inception, we have incurred significant operating losses. Our net losses were \$11.6 million and \$7.0 million for the three months ended March 31, 2015 and 2014, respectively. As of March 31, 2015, we had an accumulated deficit of \$93.8 million. We expect to continue to incur significant expenses and operating losses over the next several years. We anticipate that our expenses will increase significantly in connection with our ongoing activities, as we:

- complete IND enabling activities and commence the planned clinical development activities for our lead drug candidates BLU-285 and BLU-554;
- continue to discover, validate and develop additional drug candidates;
- maintain, expand and protect our intellectual property portfolio;
- hire additional research, development and business personnel; and

- incur additional costs associated with operating as a public company.

## **Financial Operations Overview**

### ***Revenue***

To date, we have not generated any revenue from drug sales and do not expect to generate any revenue from the sale of drugs in the near future. Our revenue consists of collaboration revenue under our agreement with Alexion, including amounts that are recognized related to an upfront payment and amounts due to us for research and development services. In the future, revenue may include milestone payments earned under the collaboration agreement and royalties on any net product sales. We expect that any revenue we generate will fluctuate from quarter to quarter as a result of the timing and amount of license fees, research and development reimbursements, payments for manufacturing services, and milestone and other payments.

In the future, we will seek to generate revenue from a combination of drug sales and additional strategic relationships we may enter into.

### ***Expenses***

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

Research and development expenses consist primarily of costs incurred for our research activities, including our drug discovery efforts, and the development of our drug candidates, which include:

- employee-related expenses including salaries, benefits, and stock-based compensation expense;
- expenses incurred under agreements with third parties that conduct research and development, pre-clinical activities and manufacturing on our behalf;
- the cost of consultants;
- the cost of lab supplies and pre-clinical study materials; and
- facilities, depreciation, and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance, and other operating costs.

Research and development costs are expensed as incurred. Costs for certain activities are recognized based on an evaluation of the progress to completion of specific tasks. Nonrefundable advance payments for goods or services to be received in the future for use in research and development activities are deferred and capitalized. The capitalized amounts are expensed as the related goods are delivered or the services are performed.

The successful development of our drug candidates is highly uncertain. As such, at this time, we cannot reasonably estimate or know the nature, timing and estimated costs of the efforts that will be necessary to complete the remainder of the development of these drug candidates. We are also unable to predict when, if ever, material net cash inflows will commence from our drug candidates. This is due to the numerous risks and uncertainties associated with developing drugs, including the uncertainty of:

- establishing an appropriate safety profile with IND-enabling toxicology studies;
- successful enrollment in, and completion of clinical trials;
- receipt of marketing approvals from applicable regulatory authorities;

- establishing commercial manufacturing capabilities or making arrangements with third-party manufacturers;
- obtaining and maintaining patent and trade secret protection and regulatory exclusivity for our drug candidates;
- commercializing the drug candidates, if and when approved, whether alone or in collaboration with others; and
- continued acceptable safety profile of the drugs following approval.

A change in the outcome of any of these variables with respect to the development of any of our drug candidates would significantly change the costs and timing associated with the development of that drug candidate.

Research and development activities are central to our business model. Drug candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. We expect research and development costs to increase significantly for the foreseeable future as our drug candidate development programs progress. However, we do not believe that it is possible at this time to accurately project total program-specific expenses through commercialization. There are numerous factors associated with the successful commercialization of any of our drug candidates, including future trial design and various regulatory requirements, many of which cannot be determined with accuracy at this time based on our stage of development. Additionally, future commercial and regulatory factors beyond our control will impact our clinical development programs and plans.

A significant portion of our research and development costs have been external costs, which we track on a program-by-program basis following nomination as a development candidate. Our internal research and development costs are primarily personnel-related costs, depreciation and other indirect costs. We do not track our internal research and development expenses on a program-by-program basis as they are deployed across multiple projects under development. The following table summarizes our external research and development expenses, by program for the three months ended March 31, 2015 and 2014. Pre-development candidate expenses, unallocated costs and internal research and development costs have been classified separately.

	Three Months Ended March 31,	
	2015	2014
	(in thousands)	
BLU-285 external costs	\$ 1,383	\$ 196
BLU-554 external costs	1,037	208
Pre-development candidate expenses and unallocated costs	3,669	3,088
Internal research and development costs	3,143	1,889
	<u>\$ 9,232</u>	<u>\$ 5,381</u>

The substantial increase in external costs associated with BLU-285 and BLU-554 for the three months ended March 31, 2015 over the three months ended March 31, 2014 was driven by the timing of the nomination of the programs as development candidates.

#### *General and Administrative Expenses*

General and administrative expenses consist primarily of salaries and other related costs, including stock-based compensation, for personnel in executive, finance, accounting, business development, legal and human resources functions. Stock-based compensation includes expense associated with stock-based awards issued to non-employees, including directors for non-board related services. Other significant costs include facility costs not otherwise included in research and development expenses, legal fees relating to patent and corporate matters and fees for accounting and consulting services.

We anticipate that our general and administrative expenses will increase in the future to support continued research and development activities, including the initiation of our clinical trials and increased costs of operating as a public company. These increases will likely include increased costs related to the hiring of additional personnel and fees to outside consultants, among other expenses. We also anticipate increased expenses associated with being a public company, including costs for audit, legal, regulatory and tax-related services, director and officer insurance premiums and investor relations costs.

#### *Other Income (Expense)*

Other income (expense) consists primarily of interest expense on amounts outstanding under a loan and security agreement, or Loan and Security Agreement, that we entered into with Silicon Valley Bank in May 2013, amortization of debt discount and the re-measurement gain or loss associated with the change in the fair value of the convertible preferred stock warrant liability.

#### ***Critical Accounting Policies and Estimates***

Our critical accounting policies are those policies which require the most significant judgments and estimates in the preparation of our financial statements. Management has determined that our most critical accounting policies are those relating to revenue recognition, accrued research and development expenses and stock-based compensation.

#### ***Revenue recognition***

We recognize revenue from license and collaboration agreements in accordance with Financial Accounting Standards Board, or FASB, Accounting Standards Codification, or ASC, Topic 605, *Revenue Recognition*, or ASC 605. Accordingly, revenue is recognized when all of the following criteria are met:

- Persuasive evidence of an arrangement exists;
- Delivery has occurred or services have been rendered;
- The seller's price to the buyer is fixed or determinable; and
- Collectability is reasonably assured.

Amounts received prior to satisfying the revenue recognition criteria are recognized as deferred revenue in the our balance sheets. Amounts expected to be recognized as revenue within the 12 months following the balance sheet date are classified as deferred revenue, current portion. Amounts not expected to be recognized as revenue within the 12 months following the balance sheet date are classified as deferred revenue, net of current portion.

Our revenue is currently generated through our collaboration agreement with Alexion. The terms of this agreement contain multiple elements, or deliverables, including an exclusive license to research, develop, manufacture and commercialize the licensed products and the compounds in the field in the territory, as well as research and development activities to be performed by us on behalf of Alexion related to the licensed product candidates. In addition, the terms of this agreement include payments to us of one or more of the following: a nonrefundable, upfront payment; contingent milestone payments related to specified pre-clinical milestones, development milestones and sales-based commercial milestones; fees for research and development services rendered; and royalties on commercial sales of licensed product candidates, if any. To date, we have received the upfront payment and certain research and development service payments but have not received any milestone payments or earned royalty revenue as a result of product sales.

When evaluating multiple element arrangements, we consider whether the deliverables under the arrangement represent separate units of accounting. This evaluation requires subjective determinations and requires management to make judgments about the individual deliverables and whether such deliverables are separable from the other aspects of the contractual relationship. In determining the units of accounting, management evaluates certain criteria, including whether the deliverables have standalone value, based on the consideration of the relevant facts and circumstances for each arrangement. The consideration received is allocated among the separate units of accounting using the relative selling price method, and the applicable revenue recognition criteria are applied to each of the separate units.

Deliverables are considered separate units of accounting provided that: (i) the delivered item(s) has value to the customer on a stand-alone basis and (ii) if the arrangement includes a general right of return relative to the delivered item(s), delivery or performance of the undelivered item(s) is considered probable and substantially in the control of the Company. In assessing whether an item has stand-alone value, we consider factors such as the research, manufacturing and commercialization capabilities of the collaboration partner and the availability of the associated expertise in the general marketplace. In addition, we consider whether the collaboration partner can use the other deliverable(s) for their intended purpose without the receipt of the remaining element(s), whether the value of the deliverable is dependent on the undelivered item(s) and whether there are other vendors that can provide the undelivered element(s). Our collaboration agreement with Alexion does not contain a general right of return relative to the delivered item(s).

Arrangement consideration that is fixed or determinable is allocated among the separate units of accounting using the relative selling price method. Then, the applicable revenue recognition criteria in ASC 605-25 are applied to each of the separate units of accounting in determining the appropriate period and pattern of recognition. We determine the selling price of a unit of accounting following the hierarchy of evidence prescribed by ASC 605-25. Accordingly, we determine the estimated selling price for units of accounting within each arrangement using vendor-specific objective evidence (VSOE) of selling price, if available, third-party evidence (TPE) of selling price if VSOE is not available, or best estimate of selling price (BESP) if neither VSOE nor TPE is available. We typically use BESP to estimate the selling price, since it generally does not have VSOE or TPE of selling price for its units of accounting. Determining the BESP for a unit of accounting requires significant judgment. In developing the BESP for a unit of accounting, we consider applicable market conditions and relevant entity-specific factors, including factors that were contemplated in negotiating the agreement with the customer and estimated costs. We validate the BESP for units of accounting by evaluating whether changes in the key assumptions used to determine the BESP will have a significant effect on the allocation of arrangement consideration between multiple units of accounting.

In the event that an element of a multiple element arrangement does not represent a separate unit of accounting, we recognize revenue from the combined element over the period over which we expect to fulfill its performance obligations or as undelivered items are delivered, as appropriate, if all of the other revenue recognition criteria in ASC 605-25 are met. If the pattern of performance in which the service is provided to the customer can be determined and objectively measurable performance measures exist, then we recognize revenue under the arrangement using the proportional performance method. If there is no discernible pattern of performance and/or objectively measurable performance measures do not exist, then we recognize revenue under the arrangement on a straight-line basis over the period the we are expected to complete our performance obligations. Revenue recognized is limited to the lesser of the cumulative amount of payments received or the cumulative amount of revenue earned, as determined using the straight-line method or proportional performance method, as applicable, as of the period ending date.

Our multiple-element revenue arrangements may include the following

#### *Exclusive Licenses*

The deliverables under our collaboration agreements may include exclusive licenses to research, develop, manufacture and commercialize licensed products. To account for this element of an arrangement, management evaluates whether an exclusive license has stand-alone value from the undelivered elements based on the consideration of the relevant facts and circumstances of the arrangement, including the research and development capabilities of the collaboration partner. We may recognize the arrangement consideration allocated to licenses upon delivery of the license if facts and circumstances indicate that the license has stand-alone value from the undelivered elements, which generally include research and development services. We defer arrangement consideration allocated to licenses if facts and circumstances indicate that the delivered license does not have stand-alone value from the undelivered elements.

When management believes a license does not have stand-alone value from the other deliverables to be provided in the arrangement, we recognize revenue attributed to the license on a proportional basis over our contractual or estimated performance period, which is typically the term of our research and development obligations. If management cannot reasonably estimate when our performance obligation ends, then revenue is deferred until management can reasonably estimate when the performance obligation ends. The periods over which revenue should be recognized are subject to estimates by management and may change over the course of the research and development and



licensing agreement. Such a change could have a material impact on the amount of revenue the Company records in future periods.

#### *Research and Development Services*

The deliverables under our collaboration agreements may include research and development services to be performed by us on behalf of the partner. Payments or reimbursements resulting from our research and development efforts are recognized as the services are performed and presented on a gross basis because we are the principal for such efforts, so long as there is persuasive evidence of an arrangement, the fee is fixed or determinable, and collection of the related amount is reasonably assured.

#### *Milestone Revenue*

Our collaboration agreements may include contingent milestone payments related to specified pre-clinical milestones, development milestones and sales-based commercial milestones.

At the inception of an arrangement that includes milestone payments, we evaluate whether each milestone is substantive and at risk to both parties on the basis of the contingent nature of the milestone. This evaluation includes an assessment of whether:

- the consideration is commensurate with either our performance to achieve the milestone or the enhancement of the value of the delivered item(s) as a result of a specific outcome resulting from the our performance to achieve the milestone;
- the consideration relates solely to past performance; and
- the consideration is reasonable relative to all of the deliverables and payment terms within the arrangement.

We evaluate factors such as the scientific, clinical, regulatory, commercial and other risks that must be overcome to achieve the respective milestone and the level of effort and investment required to achieve the respective milestone in making this assessment. There is considerable judgment involved in determining whether a milestone satisfies all of the criteria required to conclude that a milestone is substantive. Milestones that are not considered substantive are accounted for as license payments and recognized over the remaining period of performance from the date of achievement of the milestone. Milestones that are considered substantive will be recognized in their entirety upon successful accomplishment of the milestone, assuming all other revenue recognition criteria are met.

#### *Royalty Revenue*

We will recognize royalty revenue in the period of sale of the related product(s), based on the underlying contract terms, provided that the reported sales are reliably measurable and the Company has no remaining performance obligations, assuming all other revenue recognition criteria are met.

There have been no significant changes to our accounting policies discussed in our Prospectus related to accrued research and development expenses and stock-based compensation.

## Results of Operations

### Comparison of Three Months Ended March 31, 2015 and 2014

The following table summarizes our results of operations for the three months ended March 31, 2015 and 2014, together with the changes in those items in dollars and as a percentage:

	Three months ended		Dollar Change	% Change
	March 31,			
	2015	2014		
	(in thousands)			
Collaboration revenue	\$ 652	\$ —	\$ 652	100 %
Operating expenses:				
Research and development	9,232	5,381	3,851	72
General and administrative	2,770	1,572	1,198	76
Total operating expenses	<u>12,002</u>	<u>6,953</u>	<u>5,049</u>	<u>73</u>
Other income (expense):				
Other income (expense), net	(37)	18	(55)	(306)
Interest expense	(185)	(92)	(93)	(101)
Total other income (expense)	<u>(222)</u>	<u>(74)</u>	<u>(148)</u>	<u>(200)</u>
Net loss	<u>\$ (11,572)</u>	<u>\$ (7,027)</u>	<u>\$ (4,545)</u>	<u>(65)%</u>

#### Collaboration revenue

Collaboration revenue was \$0.7 million for the three months ended March 31, 2015 under our agreement with Alexion. We did not record any collaboration revenue during the three months ended March 31, 2014.

#### Research and Development Expense

Research and development expense increased \$3.8 million from \$5.4 million for the three months ended March 31, 2014 to \$9.2 million for the three months ended March 31, 2015, an increase of 72%. The increase in research and development expense was primarily attributable to the following:

- approximately \$1.4 million primarily due to an increase of 28% in headcount, largely driven by growth in the non-clinical and clinical organizations as our programs advance towards clinical trials as well as higher stock-based compensation expense;
- approximately \$1.3 million for external IND-enabling pre-clinical and toxicology studies as well as manufacturing activities for our two lead programs;
- approximately \$0.6 million in clinical activities as we advance our two lead programs towards clinical trials; and
- approximately \$0.5 million as we continue to build our platform and advance our discovery pipeline forward.

#### General and Administrative Expense

General and administrative expense increased by \$1.2 million from \$1.6 million for the three months ended March 31, 2014 to \$2.8 million for the three months ended March 31, 2015, an increase of 76%. The increase in general and administrative expense was primarily attributable to the following:

- approximately \$0.7 million in increased personnel costs primarily due to an increase of 66% in business personnel headcount to support the Company's overall growth as a publicly traded entity as well as an increase in stock-based compensation expense; and
- approximately \$0.3 million in increase in professional fees including external legal fees, corporate communications and public relations costs.

We expect that our general and administrative expense will increase in future periods as we expand our operations and incur additional costs in connection with being a public company. These increases will likely include legal, auditing and filing fees, additional insurance premiums and general compliance and consulting expenses.

#### *Other Income (Expense), Net*

Other expense increased by \$0.06 million to less than \$0.04 million for the three months ended March 31, 2015 from less than \$0.02 million of income for the three months ended March 31, 2014. The increase in other expense was primarily related to the impact of the re-measurement associated with the change in the fair value of the convertible preferred stock warrant liability.

#### *Interest Expense*

Interest expense increased by \$0.1 million to \$0.2 million for the three months ended March 31, 2015 from \$0.1 million for the three months ended March 31, 2014. The increase in interest expense was primarily related to a higher outstanding principal balance under the Loan and Security Agreement for the three months ended March 31, 2015.

### **Liquidity and Capital Resources**

#### ***Sources of Liquidity***

Through March 31, 2015, we financed our operations primarily through gross proceeds of \$115.1 million from private placements of our convertible preferred stock and proceeds of \$10.0 million from the Loan and Security Agreement. On May 5, 2015, we completed the sale of 9,367,708 shares of our common stock (inclusive of 1,221,874 shares of common stock sold by the Company pursuant to the full exercise of an option to purchase additional shares granted to the underwriters in connection with the offering) in our IPO at a price to the public of \$18.00 per share, resulting in net proceeds of \$154.2 million after deducting underwriting discounts and commissions and estimated offering costs payable by us.

As of March 31, 2015, we had cash and cash equivalents of \$50.3 million.

### Cash Flows

The following table provides information regarding our cash flows for the three months ended March 31, 2015 and 2014:

(in thousands)	Three Months Ended	
	March 31,	
	2015	2014
Net cash provided by (used in) operating activities	\$ 4,890	\$ (6,610)
Net cash used in investing activities	(1,285)	(94)
Net cash provided by (used in) financing activities	(547)	24,986
Net increase in cash and cash equivalents	\$ 3,058	\$ 18,282

Net cash provided by operating activities was \$4.9 million during the three months ended March 31, 2015 compared to net cash used in operating activities of \$6.6 million during the three months ended March 31, 2014. The increase in cash provided by operating activities was primarily due to the receipt of the upfront payment from Alexion, partially offset by an increase in net loss of \$4.5 million for the three months ended March 31, 2015 as compared to the three months ended March 31, 2014.

Net cash used in investing activities was \$1.3 million during the three months ended March 31, 2015 compared to net cash used in investing activities of \$0.1 million during the three months ended March 31, 2014. Net cash used in investing activities for the three months ended March 31, 2015 consisted of a security deposit payment for the new office lease agreement entered into by the Company as well as purchases of property and equipment. Net cash used in investing activities for the three months ended March 31, 2014 consisted of purchases of property and equipment.

Net cash used in financing activities was \$0.5 million during the three months ended March 31, 2015 compared to net cash provided by financing activities of \$24.9 million during the three months ended March 31, 2014. The cash used in financing activities for the three months ended March 31, 2015 was primarily the result of principal payments under our Loan and Security Agreement. The cash provided by financing activities for the three months ended March 31, 2014 was the result of \$24.9 million of net proceeds received from the private placement of our Series B convertible preferred stock.

### Funding Requirements

We expect our expenses to increase in connection with our ongoing activities, particularly as we continue the research and development of, initiate clinical trials of, and seek marketing approval for, our drug candidates. In addition, if we obtain marketing approval for any of our drug candidates, we expect to incur significant commercialization expenses related to drug sales, marketing, manufacturing and distribution to the extent that such sales, marketing and distribution are not the responsibility of potential collaborators. Furthermore, we expect to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we would be forced to delay, reduce or eliminate our research and development programs or future commercialization efforts.

We expect that our existing cash and cash equivalents, along with proceeds from our IPO which closed on April 29, 2015, will enable us to fund our operating expenses and capital expenditure requirements through at least early 2017. Our future capital requirements will depend on many factors, including:

- the scope, progress, results and costs of drug discovery, pre-clinical development, laboratory testing and clinical trials for our drug candidates;
- the scope, prioritization and number of our research and development programs;
- the costs, timing and outcome of regulatory review of our drug candidates;

- our ability to establish and maintain collaborations on favorable terms, if at all;
- the achievement of milestones or occurrence of other developments that trigger payments under any collaboration agreements we obtain;
- the extent to which we are obligated to reimburse, or entitled to reimbursement of, clinical trial costs under future collaboration agreements, if any;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- the extent to which we acquire or in-license other drug candidates and technologies;
- the costs of securing manufacturing arrangements for commercial production; and
- the costs of establishing or contracting for sales and marketing capabilities if we obtain regulatory approvals to market our drug candidates.

Identifying potential drug candidates and conducting pre-clinical studies and clinical trials is a time-consuming, expensive and uncertain process that takes many years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve drug sales. In addition, our drug candidates, if approved, may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of drugs that we do not expect to be commercially available for many years, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all.

Until such time, if ever, as we can generate substantial drug revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and licensing arrangements. At this time, we do not have any committed external source of funds outside of those to be earned in connection with our agreement with Alexion. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a common stockholder. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends.

If we raise funds through additional collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or drug candidates or to grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our drug development or future commercialization efforts or grant rights to develop and market drug candidates that we would otherwise prefer to develop and market ourselves.

#### **Contractual Obligations**

There have been no material changes to our contractual obligations and commitments from those described under “Management’s Discussion and Analysis of Financial Condition and Results of Operations” included in the Prospectus.

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

We did not have, during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined under applicable Securities and Exchange Commission rules.

### **Item 3. Quantitative and Qualitative Disclosures About Market Risk**

We are exposed to market risk related to changes in interest rates. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because our investments, including cash and cash equivalents, are in a money market fund that invests in U.S. Treasury obligations.

We are also exposed to market risk related to changes in foreign currency exchange rates. From time to time we contract with vendors that are located Asia and Europe, which are denominated in foreign currencies. We are subject to fluctuations in foreign currency rates in connection with these agreements. We do not currently hedge our foreign currency exchange rate risk. As of March 31, 2015 and 2014, we had minimal or no liabilities denominated in foreign currencies.

Inflation generally affects us by increasing our cost of labor and clinical trial costs. We do not believe that inflation had a material effect on our business, financial condition or results of operations during the three months ended March 31, 2015 and 2014.

### **Item 4. Controls and Procedures**

#### *Management's Evaluation of our Disclosure Controls and Procedures*

We maintain "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act, that are designed to ensure that information required to be disclosed in the reports that we file or submit under the Exchange Act is (1) recorded, processed, summarized and reported, within the time periods specified in the Securities and Exchange Commission's rules and forms and (2) accumulated and communicated to our management, including our principal executive and principal financial officer, as appropriate to allow timely decisions regarding required disclosure. Our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and our management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Our disclosure controls and procedures are designed to provide reasonable assurance of achieving their control objectives.

Our management, with the participation of our Chief Executive Officer and Chief Business / Principal Accounting and Finance Officer, has evaluated the effectiveness of our disclosure controls and procedures as of March 31, 2015, the end of the period covered by this Quarterly Report on Form 10-Q. Based upon such evaluation, our Chief Executive Officer and Chief Business Officer / Principal Accounting and Finance Officer have concluded that our disclosure controls and procedures were effective at the reasonable assurance level as of such date.

#### *Changes in Internal Control over Financial Reporting*

There was no change in our internal control over financial reporting that occurred during the period covered by this Quarterly Report on Form 10-Q that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

## Part II – OTHER INFORMATION

### Item 1. Legal Proceedings

We are not currently a party to any material legal proceedings.

### Item 1A. Risk Factors

*Investing in our common stock involves a high degree of risk. You should carefully consider the following risks and uncertainties, together with all other information in this Quarterly Report on Form 10-Q, including our financial statements and related notes, before investing in our common stock. Any of the risk factors we describe below could adversely affect our business, financial condition or results of operations. The market price of our common stock could decline if one or more of these risks or uncertainties actually occur, causing you to lose all or part of the money you paid to buy our common stock. Additional risks that we currently do not know about or that we currently believe to be immaterial may also impair our business.*

#### **Risks Related to Our Financial Position and Need for Additional Capital**

***We are a biopharmaceutical company with a limited operating history and have not generated any revenue from drug sales. We have incurred significant operating losses since our inception and anticipate that we will incur continued losses for the foreseeable future.***

We are a biopharmaceutical company with a limited operating history on which to base your investment decision. Biopharmaceutical drug development is a highly speculative undertaking and involves a substantial degree of risk. We commenced operations in April 2011. Our operations to date have been limited primarily to organizing and staffing our company, business planning, raising capital, developing our technology, identifying potential drug candidates and undertaking pre-clinical studies of our most advanced drug candidates. We have only recently identified lead drug candidates for two of our programs. We have never generated any revenue from drug sales. We have not obtained regulatory approvals for any of our drug candidates.

We have not yet demonstrated our ability to initiate or successfully complete any clinical trials, including large-scale, pivotal clinical trials, obtain regulatory approvals, manufacture a commercial scale drug, or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful commercialization. Typically, it takes many years to develop one new drug from the time it is discovered to when it is available for treating patients. Consequently, any predictions you make about our future success or viability may not be as accurate as they could be if we had a longer operating history. In addition, as a new business, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors. We will need to transition from a company with a research focus to a company capable of supporting commercial activities. We may not be successful in such a transition.

Since inception, we have focused substantially all of our efforts and financial resources on developing our proprietary compound library, novel target discovery engine and initial drug candidates. We have funded our operations to date through proceeds from sales of convertible preferred stock and, to a lesser extent, through a loan and security agreement, or Loan and Security Agreement, that we entered into with Silicon Valley Bank in May 2013. Through March 31, 2015, we raised an aggregate of \$125.1 million of gross proceeds from such transactions. As of March 31, 2015, our cash and cash equivalents and investments were \$50.3 million. On May 5, 2015, we completed the sale of 9,367,708 shares of our common stock (inclusive of 1,221,874 shares of common stock sold by the Company pursuant to the full exercise of an option to purchase additional shares granted to the underwriters in connection with the offering) in our IPO at a price to the public of \$18.00 per share, resulting in net proceeds of \$154.2 million after deducting underwriting discounts and commissions and estimated offering costs payable by us. We have incurred net losses in each year since our inception, and we had an accumulated deficit of \$93.8 million as of March 31, 2015. Our net losses were \$11.6 million and \$7.0 million for the three months ended March 31, 2015 and 2014, respectively. Substantially all of our operating losses have resulted from costs incurred in connection with our research and development programs and

from general and administrative costs associated with our operations. We expect to continue to incur significant expenses and operating losses over the next several years and for the foreseeable future. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our stockholders' deficit and working capital. We expect our research and development expenses to significantly increase in connection with beginning clinical trials of our drug candidates. In addition, if we obtain marketing approval for our drug candidates, we will incur significant sales, marketing and outsourced-manufacturing expenses. As a public company, we will incur additional costs associated with operating as a public company. As a result, we expect to continue to incur significant and increasing operating losses for the foreseeable future. Because of the numerous risks and uncertainties associated with developing pharmaceutical drugs, we are unable to predict the extent of any future losses or when we will become profitable, if at all. Even if we do become profitable, we may not be able to sustain or increase our profitability on a quarterly or annual basis. Our ability to become profitable depends upon our ability to generate revenue. To date, we have not generated any revenue from our lead drug candidates, BLU-285 and BLU-554, and we do not know and do not expect to generate any revenue from the sale of drugs in the near future. We do not expect to generate significant revenue unless and until we obtain marketing approval of, and begin to sell, BLU-285, BLU-554 or one of our other drug candidates. Our ability to generate revenue depends on a number of factors, including, but not limited to, our ability to:

- initiate and successfully complete clinical trials that meet their clinical endpoints;
- initiate and successfully complete all safety studies required to obtain U.S. and foreign marketing approval for our drug candidates;
- commercialize our drug candidates, if approved, by developing a sales force or entering into additional collaborations with third parties; and
- achieve market acceptance of our drug candidates in the medical community and with third-party payors.

We expect to incur significant sales and marketing costs as we prepare to commercialize our drug candidates. Even if we initiate and successfully complete pivotal clinical trials of our drug candidates, and our drug candidates are approved for commercial sale, and despite expending these costs, our drug candidates may not be commercially successful. We may not achieve profitability soon after generating drug sales, if ever. If we are unable to generate drug revenue, we will not become profitable and may be unable to continue operations without continued funding.

***We may need to raise substantial additional funding. If we are unable to raise capital when needed, we would be forced to delay, reduce or eliminate some of our drug development programs or commercialization efforts.***

The development of pharmaceutical drugs is capital-intensive. We are currently advancing our drug candidates through pre-clinical development and anticipate beginning clinical trials for our lead drug candidates, BLU-285 and BLU-554, in mid-2015. We expect our expenses to increase in connection with our ongoing activities, particularly as we continue the research and development of, initiate clinical trials of, and seek marketing approval for, our drug candidates. In addition, depending on the status of regulatory approval or, if we obtain marketing approval for any of our drug candidates, we expect to incur significant commercialization expenses related to drug sales, marketing, manufacturing and distribution to the extent that such sales, marketing and distribution are not the responsibility of Alexion Pharma Holding, or Alexion, or other collaborators. We may also need to raise additional funds sooner if we choose to pursue additional indications and/or geographies for our drug candidates or otherwise expand more rapidly than we presently anticipate. Furthermore, upon the closing of this offering, we expect to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we would be forced to delay, reduce or eliminate certain of our research and development programs or future commercialization efforts.



We expect that the net proceeds from our IPO, together with our existing cash and cash equivalents, will be sufficient to fund our operations through at least early 2017. Our future capital requirements will depend on and could increase significantly as a result of many factors, including:

- the scope, progress, results and costs of drug discovery, pre-clinical development, laboratory testing and clinical trials for our drug candidates;
- the scope, prioritization and number of our research and development programs;
- the success of our collaboration with Alexion;
- the costs, timing and outcome of regulatory review of our drug candidates;
- our ability to establish and maintain additional collaborations on favorable terms, if at all;
- the achievement of milestones or occurrence of other developments that trigger payments under any additional collaboration agreements we obtain;
- the extent to which we are obligated to reimburse, or entitled to reimbursement of, clinical trial costs under future collaboration agreements, if any;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- the extent to which we acquire or in-license other drug candidates and technologies.
- the costs of securing manufacturing arrangements for commercial production; and
- the costs of establishing or contracting for sales and marketing capabilities if we obtain regulatory clearances to market our drug candidates.

Identifying potential drug candidates and conducting pre-clinical development testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve drug sales. In addition, our drug candidates, if approved, may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of drugs that we do not expect to be commercially available for many years, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives.

Any additional fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize our drug candidates. Dislocations in the financial markets have generally made equity and debt financing more difficult to obtain, and may have a material adverse effect on our ability to meet our fundraising needs. We cannot guarantee that future financing will be available in sufficient amounts or on terms acceptable to us, if at all. Moreover, the terms of any financing may adversely affect the holdings or the rights of our stockholders and the issuance of additional securities, whether equity or debt, by us, or the possibility of such issuance, may cause the market price of our shares to decline. The sale of additional equity or convertible securities would dilute all of our stockholders. The incurrence of indebtedness would result in increased fixed payment obligations and we may be required to agree to certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. We could also be required to seek funds through arrangements with collaborators or otherwise at an earlier stage than otherwise would be desirable and we may be required to relinquish rights to some of our technologies or drug candidates or otherwise agree to terms unfavorable to us, any of which may have a material adverse effect on our business, operating results and prospects.

If we are unable to obtain funding on a timely basis, we may be required to significantly curtail, delay or discontinue one or more of our research or development programs or the commercialization of any drug candidate or be unable to expand our operations or otherwise capitalize on our business opportunities, as desired, which could materially affect our business, financial condition and results of operations.

***Raising additional capital may cause dilution to our stockholders, including purchasers of common stock in this offering, restrict our operations or require us to relinquish rights to our technologies or drug candidates.***

Until such time, if ever, as we can generate substantial drug revenues, we expect to finance our cash needs through a combination of private and public equity offerings, debt financings, collaborations, strategic alliances and licensing arrangements. We do not have any committed external source of funds, other than our collaboration with Alexion, which is limited in scope and duration, and funds already borrowed under the Loan and Security Agreement. To the extent that we raise additional capital through the sale of common stock or securities convertible or exchangeable into common stock, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that materially adversely affect your rights as a common stockholder. Debt financing, if available, would increase our fixed payment obligations and may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends.

If we raise funds through additional collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our intellectual property, future revenue streams, research programs or drug candidates or to grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our drug development or future commercialization efforts or grant rights to develop and market drug candidates that we would otherwise prefer to develop and market ourselves.

### **Risks Related to Drug Development and Regulatory Approval**

***We are very early in our development efforts. All of our lead drug candidates are still in pre-clinical development. If we are unable to advance our drug candidates to clinical development, obtain regulatory approval and ultimately commercialize our drug candidates or experience significant delays in doing so, our business will be materially harmed.***

We are very early in our development efforts, and all of our lead drug candidates are still in pre-clinical development. We have only recently identified lead drug candidates for two of our programs. We have invested substantially all of our efforts and financial resources in the identification and pre-clinical development of kinase inhibitors, including the development of our lead drug candidates, BLU-285 and BLU-554. Our ability to generate drug revenues, which we do not expect will occur for many years, if ever, will depend heavily on the successful development and eventual commercialization of our drug candidates, which may never occur. We currently generate no revenues from sales of any drugs, and we may never be able to develop or commercialize a marketable drug. Each of our drug candidates will require additional pre-clinical and clinical development, management of clinical, pre-clinical and manufacturing activities, regulatory approval in multiple jurisdictions, obtaining manufacturing supply, building of a commercial organization, substantial investment and significant marketing efforts before we generate any revenues from drug sales. In addition, our drug development programs contemplate the development of companion diagnostics, which are assays or tests to identify an appropriate patient population. Companion diagnostics are subject to regulation as medical devices and must themselves be approved for marketing by the U.S. Food and Drug Administration, or FDA, or certain other foreign regulatory agencies before we may commercialize our drug candidates. The success of our drug candidates will depend on several factors, including the following:

- successful completion of pre-clinical studies;
- approval of Investigational New Drug applications, or INDs, for our planned clinical trials or future clinical trials;
- successful enrollment in, and completion of, clinical trials;
- successful development of companion diagnostics for use with our drug candidates;

- receipt of regulatory approvals from applicable regulatory authorities;
- establishing commercial manufacturing capabilities or making arrangements with third-party manufacturers for clinical supply and commercial manufacturing;
- obtaining and maintaining patent and trade secret protection or regulatory exclusivity for our drug candidates;
- launching commercial sales of our drug candidates, if and when approved, whether alone or in collaboration with others;
- acceptance of the drug candidates, if and when approved, by patients, the medical community and third party payors;
- effectively competing with other therapies;
- obtaining and maintaining healthcare coverage and adequate reimbursement;
- enforcing and defending intellectual property rights and claims; and
- maintaining a continued acceptable safety profile of the drug candidates following approval.

If we do not achieve one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize our drug candidates, which would materially harm our business. If we do not receive regulatory approvals for our drug candidates, we may not be able to continue our operations.

***Our approach to the discovery and development of drug candidates that inhibit kinases is unproven, and we do not know whether we will be able to develop any drugs of commercial value.***

Our scientific approach focuses on using our novel target discovery engine and our proprietary compound library to identify new kinase targets in disease indications. Our focus on using our novel target discovery engine to identify potential kinase targets in disease indications may not result in the discovery and development of commercially viable drugs for these diseases. The use of our proprietary compound library may not lead to the development of commercially viable drugs. Even if we are able to develop a drug candidate that successfully targets these kinases in pre-clinical studies, we may not succeed in demonstrating safety and efficacy of the drug candidate in clinical trials.

***Clinical drug development involves a lengthy and expensive process, with an uncertain outcome. We may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of our drug candidates.***

All of our lead drug candidates are in pre-clinical development and their risk of failure is high. It is impossible to predict when or if any of our drug candidates will prove effective and safe in humans or will receive regulatory approval. Before obtaining marketing approval from regulatory authorities for the sale of any drug candidate, we must complete pre-clinical studies and then conduct extensive clinical trials to demonstrate the safety and efficacy of our drug candidates in humans. Clinical testing is expensive, difficult to design and implement, can take many years to complete and is uncertain as to outcome. A failure of one or more clinical trials can occur at any stage of testing. The outcome of pre-clinical development testing and early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results. Moreover, pre-clinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their drug candidates performed satisfactorily in pre-clinical studies and clinical trials have nonetheless failed to obtain marketing approval of their product candidates. Our pre-clinical studies and future clinical trials may not be successful.

We plan to commence a Phase 1 clinical trial of BLU-285 as a treatment for systemic mastocytosis, or SM, a Phase 1 clinical trial of BLU-285 as a treatment for gastrointestinal stromal tumor, or GIST, and a Phase 1 clinical trial of BLU-554 as a treatment for hepatocellular carcinoma, or HCC. Commencing each of these clinical trials is subject to finalizing the trial design based on ongoing discussions with the FDA and other regulatory authorities. For example,

based on our ongoing pre-IND discussions relating to our BLU-285 program, we may be required to submit data from our ongoing 13-week toxicology studies to further support our recommended safe starting dose and define the dose limiting toxicity. In the event that the FDA requires us to submit data from such studies or we are required to satisfy other FDA requests, the start of our clinical trials for BLU-285 may be delayed. Even after we receive and incorporate guidance from these regulatory authorities, the FDA or other regulatory authorities could disagree that we have satisfied their requirements to commence our clinical trials or change their position on the acceptability of our trial designs or the clinical endpoints selected, which may require us to complete additional pre-clinical studies or clinical trials or impose stricter approval conditions than we currently expect. Successful completion of our clinical trials is a prerequisite to submitting a new drug application, or NDA, to the FDA and a Marketing Authorization Application, or MAA, in Europe for each drug candidate and, consequently, the ultimate approval and commercial marketing of BLU-285, BLU-554 and our other drug candidates. We do not know whether any of our clinical trials will begin or be completed on schedule, if at all.

We may experience delays in completing our pre-clinical studies and initiating or completing clinical trials, and we may experience numerous unforeseen events during, or as a result of, any future clinical trials that we could conduct that could delay or prevent our ability to receive marketing approval or commercialize our drug candidates, including:

- regulators or institutional review boards, or IRBs, or ethics committees may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- we may experience delays in reaching, or fail to reach, agreement on acceptable terms with prospective trial sites and prospective contract research organizations, or CROs, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- clinical trials of our drug candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional pre-clinical studies or clinical trials or we may decide to abandon drug development programs;
- the number of patients required for clinical trials of our drug candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate or participants may drop out of these clinical trials or fail to return for post-treatment follow-up at a higher rate than we anticipate;
- our third party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all, or may deviate from the clinical trial protocol or drop out of the trial, which may require that we add new clinical trial sites or investigators;
- we may elect to, or regulators or IRBs or ethics committees may require that we or our investigators suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks;
- the cost of clinical trials of our drug candidates may be greater than we anticipate;
- the supply or quality of our drug candidates or other materials necessary to conduct clinical trials of our drug candidates may be insufficient or inadequate;
- our drug candidates may have undesirable side effects or other unexpected characteristics, causing us or our investigators, regulators or IRBs or ethics committees to suspend or terminate the trials, or reports may arise from pre-clinical or clinical testing of other cancer therapies that raise safety or efficacy concerns about our drug candidates; and
- the FDA or other regulatory authorities may require us to submit additional data or impose other requirements before permitting us to initiate a clinical trial.

We could encounter delays if a clinical trial is suspended or terminated by us, by the IRBs of the institutions in which such trials are being conducted, by the Data Safety Monitoring Board, or DSMB, for such trial or by the FDA or other regulatory authorities. Such authorities may impose such a suspension or termination due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial. Many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our drug candidates. Further, the FDA may disagree with our clinical trial design and our interpretation of data from clinical trials, or may change the requirements for approval even after it has reviewed and commented on the design for our clinical trials.

If we are required to conduct additional clinical trials or other testing of our drug candidates beyond those that we currently contemplate, if we are unable to successfully complete clinical trials of our drug candidates or other testing, if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we may:

- be delayed in obtaining marketing approval for our drug candidates;
- not obtain marketing approval at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- be subject to post-marketing testing requirements; or
- have the drug removed from the market after obtaining marketing approval.

Our drug development costs will also increase if we experience delays in testing or regulatory approvals. We do not know whether any of our clinical trials will begin as planned, will need to be restructured or will be completed on schedule, or at all. Significant pre-clinical study or clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our drug candidates or allow our competitors to bring products to market before we do and impair our ability to successfully commercialize our drug candidates and may harm our business and results of operations. Any delays in our pre-clinical or future clinical development programs may harm our business, financial condition and prospects significantly.

***If we experience delays or difficulties in the enrollment of patients in clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented.***

We may not be able to initiate or continue clinical trials for our drug candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA or similar regulatory authorities outside the United States. In particular, because we are focused on patients with genomically defined diseases, our ability to enroll eligible patients may be limited or may result in slower enrollment than we anticipate. In addition, some of our competitors have ongoing clinical trials for drug candidates that treat the same indications as our drug candidates, and patients who would otherwise be eligible for our clinical trials may instead enroll in clinical trials of our competitors' drug candidates.

Patient enrollment may be affected by other factors including:

- the severity of the disease under investigation;
- the eligibility criteria for the clinical trial in question;
- the availability of an appropriate genomic screening test;

- the perceived risks and benefits of the drug candidate under study;
- the efforts to facilitate timely enrollment in clinical trials;
- the patient referral practices of physicians;
- the ability to monitor patients adequately during and after treatment; and
- the proximity and availability of clinical trial sites for prospective patients.

***Genomically defined diseases may have relatively low prevalence and it may be difficult to identify patients with the genomic driver of the disease, which may lead to delays in enrollment for our trials.***

Following our general drug development strategy, we have designed our planned Phase 1 clinical trials of each of BLU-285 and BLU-554, and expect to design future trials, to include some patients with the applicable genomic alteration that causes the disease with a view to assessing possible early evidence of potential therapeutic effect. Genomically defined diseases, however, may have relatively low prevalence and it may be difficult to identify patients with the applicable genomic alteration. We intend to engage third parties to develop companion diagnostics for use in our clinical trials, but such third parties may not be successful in developing such companion diagnostics, furthering the difficulty in identifying patients with the applicable genomic alteration for our clinical trials. Our inability to enroll a sufficient number of patients with the applicable genomic alteration for our clinical trials would result in significant delays and could require us to abandon one or more clinical trials altogether. Enrollment delays in our clinical trials may result in increased development costs for our drug candidates, which would cause the value of our company to decline and limit our ability to obtain additional financing. Further, if we are unable to include patients with the applicable genomic alteration, this could compromise our ability to seek participation in FDA's expedited review and approval programs, including Breakthrough Therapy Designation and Fast Track Designation, or otherwise to seek to accelerate clinical development and regulatory timelines.

***If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals both for our drug candidates and for the related companion diagnostics, we will not be able to commercialize, or will be delayed in commercializing, our drug candidates, and our ability to generate revenue will be materially impaired.***

Our drug candidates and the related companion diagnostics and the activities associated with their development and commercialization, including their design, testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale, distribution, import and export are subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and by comparable authorities in other countries. Before we can commercialize any of our drug candidates, we must obtain marketing approval. We may also need marketing approval for the related companion diagnostics. We have not received approval to market any of our drug candidates or related companion diagnostics from regulatory authorities in any jurisdiction and it is possible that none of our drug candidates or any drug candidates or related companion diagnostics we may seek to develop in the future will ever obtain regulatory approval. We have only limited experience in filing and supporting the applications necessary to gain regulatory approvals and expect to rely on third-party CROs and/or regulatory consultants to assist us in this process. Securing regulatory approval requires the submission of extensive pre-clinical and clinical data and supporting information to the various regulatory authorities for each therapeutic indication to establish the drug candidate's safety and efficacy. Securing regulatory approval also requires the submission of information about the drug manufacturing process to, and inspection of manufacturing facilities by, the relevant regulatory authority. Our drug candidates may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our obtaining marketing approval or prevent or limit commercial use.

The process of obtaining regulatory approvals, both in the United States and abroad, is expensive, may take many years if additional clinical trials are required, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the drug candidates involved. Changes in marketing approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted NDA, Pre-Market Approval, or PMA, or equivalent application types,

may cause delays in the approval or rejection of an application. The FDA and comparable authorities in other countries have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional pre-clinical, clinical or other studies. Our drug candidates could be delayed in receiving, or fail to receive, regulatory approval for many reasons, including the following:

- the FDA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials;
- we may be unable to demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities that a drug candidate is safe and effective for its proposed indication or a related companion diagnostic is suitable to identify appropriate patient populations;
- the results of clinical trials may not meet the level of statistical significance required by the FDA or comparable foreign regulatory authorities for approval;
- we may be unable to demonstrate that a drug candidate's clinical and other benefits outweigh its safety risks;
- the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from pre-clinical studies or clinical trials;
- the data collected from clinical trials of our drug candidates may not be sufficient to support the submission of an NDA or other submission or to obtain regulatory approval in the United States or elsewhere;
- the FDA or comparable foreign regulatory authorities may fail to approve the manufacturing processes or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; and
- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

In addition, even if we were to obtain approval, regulatory authorities may approve any of our drug candidates for fewer or more limited indications than we request, may not approve the price we intend to charge for our drugs and related companion diagnostics, may grant approval contingent on the performance of costly post-marketing clinical trials, or may approve a drug candidate with a label that does not include the labeling claims necessary or desirable for the successful commercialization of that drug candidate. Any of the foregoing scenarios could materially harm the commercial prospects for our drug candidates.

If we experience delays in obtaining approval or if we fail to obtain approval of our drug candidates and related companion diagnostics, the commercial prospects for our drug candidates may be harmed and our ability to generate revenues will be materially impaired.

***Our drug candidates may cause undesirable side effects 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 drug candidates could cause us to interrupt, delay or halt pre-clinical studies or 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 regulatory authorities. While we have not yet initiated clinical trials for any of our drug candidates, as is the case with all oncology drugs, it is likely that there may be side effects associated with their use. Results of our trials could reveal a high and unacceptable severity and prevalence of these or other 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 drug candidates for any or all targeted indications. The drug-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.

Further, our drug candidates could cause undesirable side effects in clinical trials related to on-target toxicity. For example, the FGF19/FGFR4 signaling axis has been shown to play a role in the regulation of de novo bile acid synthesis. Modulation of this signaling axis by treatment with a small molecule FGFR4 inhibitor could lead to the clinical symptoms that were observed with administration of an FGF19 antibody. If on-target toxicity is observed, or if our drug candidates have characteristics that are unexpected, we may need to abandon their development or limit development to more narrow uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective. Many compounds that initially showed promise in early stage testing for treating cancer have later been found to cause side effects that prevented further development of the compound.

Further, clinical trials by their nature utilize a sample of the potential patient population. With a limited number of patients and limited duration of exposure, rare and severe side effects of our drug candidates may only be uncovered with a significantly larger number of patients exposed to the drug candidate. If our drug candidates receive marketing approval and we or others identify undesirable side effects caused by such drug candidates (or any other similar drugs) after such approval, a number of potentially significant negative consequences could result, including:

- regulatory authorities may withdraw or limit their approval of such drug candidates;
- regulatory authorities may require the addition of labeling statements, such as a “boxed” warning or a contraindication;
- we may be required to create a medication guide outlining the risks of such side effects for distribution to patients;
- we may be required to change the way such drug candidates are distributed or administered, conduct additional clinical trials or change the labeling of the drug candidates;
- regulatory authorities may require a Risk Evaluation and Mitigation Strategy, or REMS, plan to mitigate risks, which could include medication guides, physician communication plans, or elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools;
- we may be subject to regulatory investigations and government enforcement actions;
- we may decide to remove such drug candidates from the marketplace;
- we could be sued and held liable for injury caused to individuals exposed to or taking our drug candidates; and
- our reputation may suffer.

We believe that any of these events could prevent us from achieving or maintaining market acceptance of the affected drug candidates and could substantially increase the costs of commercializing our drug candidates, if approved, and significantly impact our ability to successfully commercialize our drug candidates and generate revenues.

***A Breakthrough Therapy Designation by the FDA for our drug candidates may not lead to a faster development or regulatory review or approval process, and it does not increase the likelihood that our drug candidates will receive marketing approval.***

We may seek a Breakthrough Therapy Designation for some of our drug candidates. A breakthrough therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For drugs that have been designated as breakthrough therapies,



interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Drugs designated as breakthrough therapies by the FDA are also eligible for accelerated approval.

Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe one of our drug candidates meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of a Breakthrough Therapy Designation for a drug candidate may not result in a faster development process, review or approval compared to drugs considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our drug candidates qualify as breakthrough therapies, the FDA may later decide that the drugs no longer meet the conditions for qualification.

***A Fast Track Designation by the FDA may not actually lead to a faster development or regulatory review or approval process.***

We may seek Fast Track Designation for some of our drug candidates. If a drug is intended for the treatment of a serious or life-threatening condition and the drug demonstrates the potential to address unmet medical needs for this condition, the drug sponsor may apply for Fast Track Designation. The FDA has broad discretion whether or not to grant this designation, so even if we believe a particular drug candidate is eligible for this designation, we cannot assure you that the FDA would decide to grant it. Even if we do receive Fast Track Designation, we may not experience a faster development process, review or approval compared to conventional FDA procedures. The FDA may withdraw Fast Track Designation if it believes that the designation is no longer supported by data from our clinical development program.

***We may seek Orphan Drug Designation for some of our drug candidates, and we may be unsuccessful or may be unable to maintain the benefits associated with Orphan Drug Designation, including the potential for market exclusivity.***

As part of our business strategy, we may seek Orphan Drug Designation for our drug candidates, and we may be unsuccessful. Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a drug as an orphan drug if it is a drug intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals annually in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. In the United States, Orphan Drug Designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages and user-fee waivers.

Similarly, in Europe, the European Commission grants Orphan Drug Designation after receiving the opinion of the European Medicines Agency's, or EMA, Committee for Orphan Medicinal Products on an Orphan Drug Designation application. Orphan Drug Designation is intended to promote the development of drugs that are intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions affecting not more than 5 in 10,000 persons in Europe and for which no satisfactory method of diagnosis, prevention, or treatment has been authorized (or the product would be a significant benefit to those affected). Additionally, designation is granted for drugs intended for the diagnosis, prevention, or treatment of a life-threatening, seriously debilitating or serious and chronic condition and when, without incentives, it is unlikely that sales of the drug in Europe would be sufficient to justify the necessary investment in developing the drug. In Europe, Orphan Drug Designation entitles a party to financial incentives such as reduction of fees or fee waivers.

Generally, if a drug with an Orphan Drug Designation subsequently receives the first marketing approval for the indication for which it has such designation, the drug is entitled to a period of marketing exclusivity, which precludes the EMA or the FDA from approving another marketing application for the same drug and indication for that time period, except in limited circumstances. The applicable period is seven years in the United States and ten years in Europe. The European exclusivity period can be reduced to six years if a drug no longer meets the criteria for Orphan Drug Designation or if the drug is sufficiently profitable so that market exclusivity is no longer justified.

Even if we obtain orphan drug exclusivity for a drug, that exclusivity may not effectively protect the drug from competition because different drugs can be approved for the same condition. Even after an orphan drug is approved, the FDA can subsequently approve the same drug for the same condition if the FDA concludes that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. In addition, a designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation. Moreover, orphan drug exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition. Orphan Drug Designation neither shortens the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process. While we intend to seek Orphan Drug Designation for our drug candidates, we may never receive such designations. Even if we do receive such designations, there is no guarantee that we will enjoy the benefits of those designations.

***Even if we receive regulatory approval for any of our drug candidates, we will be subject to ongoing obligations and continued regulatory review, which may result in significant additional expense. Additionally, our drug candidates, if approved, could be subject to labeling and other restrictions and market withdrawal and we may be subject to penalties if we fail to comply with regulatory requirements or experience unanticipated problems with our drugs.***

If the FDA or a comparable foreign regulatory authority approves any of our drug candidates, the manufacturing processes, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion and recordkeeping for the drug 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 current Good Manufacturing Practices, or cGMPs, and Good Clinical Practices, or GCPs, for any clinical trials that we conduct post-approval. Any regulatory approvals that we receive for our drug candidates may also be subject to limitations on the approved indicated uses for which the 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 drug. Later discovery of previously unknown problems with a drug, including adverse events of unanticipated severity or frequency, or with our third-party manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may result in, among other things:

- restrictions on the marketing or manufacturing of the drug, withdrawal of the drug from the market, or voluntary drug recalls;
- fines, warning letters or holds on clinical trials;
- refusal by the FDA to approve pending applications or supplements to approved applications filed by us, or suspension or revocation of drug license approvals;
- drug seizure or detention, or refusal to permit the import or export of drugs; and
- injunctions or the imposition of civil or criminal penalties.

The FDA's policies may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our drug 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 may not be successful in our efforts to use and expand our development platform to build a pipeline of drug candidates.***

A key element of our strategy is to use our novel target discovery engine to identify kinases that are drivers in genomically defined diseases with high unmet medical need in order to build a pipeline of drug candidates. Although our research and development efforts to date have resulted in a pipeline of drug candidates, we may not be able to continue

to identify novel kinase drivers and develop drug candidates. Even if we are successful in continuing to build our pipeline, the potential drug candidates that we identify may not be suitable for clinical development. For example, they may be shown to have harmful side effects or other characteristics that indicate that they are unlikely to be drugs that will receive marketing approval and achieve market acceptance. If we do not successfully develop and commercialize drug candidates based upon our approach, we will not be able to obtain drug revenues in future periods, which likely would result in significant harm to our financial position and adversely affect our stock price.

***We may expend our limited resources to pursue a particular drug candidate or indication and fail to capitalize on drug candidates or indications that may be more profitable or for which there is a greater likelihood of success.***

Because we have limited financial and managerial resources, we focus on research programs and drug candidates that we identify for specific indications. As a result, we may forego or delay pursuit of opportunities with other drug candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial drugs or profitable market opportunities. Our spending on current and future research and development programs and drug candidates for specific indications may not yield any commercially viable drugs. If we do not accurately evaluate the commercial potential or target market for a particular drug candidate, we may relinquish valuable rights to that drug candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such drug candidate.

#### **Risks Related to Commercialization**

***The incidence and prevalence for target patient populations of our drug candidates have not been established with precision. If the market opportunities for our drug candidates are smaller than we estimate or if any approval that we obtain is based on a narrower definition of the patient population, our revenue and ability to achieve profitability will be adversely affected, possibly materially.***

The precise incidence and prevalence for SM, GIST and HCC are unknown. Our projections of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with our drug candidates, are based on estimates. We estimate that there are approximately: (i) 4,500 addressable patients with advanced forms of SM and approximately 16,000 addressable patients with indolent SM in the United States, France, Germany, Italy, Spain, the United Kingdom and Japan, or the Major Markets; (ii) 500 addressable patients with PDGFR $\alpha$  D842V-driven, unresectable or metastatic GIST in the Major Markets and approximately 20,000 addressable patients in the Major Markets with unresectable or metastatic frontline GIST; and (iii) 18,000 first line and 6,000 second line addressable HCC patients with aberrantly active FGFR4, signaling in the Major Markets.

The total addressable market opportunity for BLU-285 for the treatment of patients with SM and GIST and BLU-554 for the treatment of HCC patients with aberrantly active FGFR4 signaling will ultimately depend upon, among other things, the diagnosis criteria included in the final label for each of BLU-285 and BLU-554, if our drug candidates are approved for sale for these indications, acceptance by the medical community and patient access, drug pricing and reimbursement. The number of patients in the Major Markets and elsewhere may turn out to be lower than expected, patients may not be otherwise amenable to treatment with our drugs, or new patients may become increasingly difficult to identify or gain access to, all of which would adversely affect our results of operations and our business.

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

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

Specifically, there are a large number of companies developing or marketing treatments for cancer, including many major pharmaceutical and biotechnology companies. If BLU-285 receives marketing approval for advanced SM, GIST and/or for patients with GIST with the PDGFR $\alpha$  D842V mutation, it may face competition from other drug candidates in development for these indications, including drug candidates in development from AB Science S.A., Plexxikon Inc., a wholly-owned subsidiary of Daiichi Sankyo Company, Limited, Deciphera Pharmaceuticals, LLC, Novartis AG, AROG Pharmaceuticals, Inc. and ARIAD Pharmaceuticals, Inc. Further, if BLU-554 receives marketing approval for patients with HCC with FGF19 overexpression, it will face competition from sorafenib, the only approved systemic medical therapy for HCC. In addition, we are aware of potentially competitive drug candidates in development by AstraZeneca plc, Bayer AG, Johnson & Johnson, Novartis AG, Taiho Pharmaceutical Co., Ltd., Celgene Corporation, Sanofi S.A., Eisai Inc., H3 Biomedicine Inc. and Xoma Ltd.

Many of the companies against which we are competing or against which we may compete in the future have significantly greater financial resources and expertise in research and development, manufacturing, pre-clinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved drugs than we do. Mergers and acquisitions in the pharmaceutical, biotechnology and diagnostic industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize drugs that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any drugs that we or our collaborators may develop. Our competitors also may obtain FDA or other regulatory approval for their drugs more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we or our collaborators are able to enter the market. The key competitive factors affecting the success of all of our drug candidates, if approved, are likely to be their efficacy, safety, convenience, price, the effectiveness of companion diagnostics in guiding the use of related drugs, the level of generic competition and the availability of reimbursement from government and other third-party payors.

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

We will face an inherent risk of product liability exposure related to the testing of our drug candidates in human clinical trials and will face an even greater risk if we commercially sell any drug candidates that we may develop. If we cannot successfully defend ourselves against claims that our drug candidates caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any drug candidates that we may develop;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend the related litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue; and
- the inability to commercialize any drug candidates that we may develop.

Although we maintain product liability insurance coverage, it may not be adequate to cover all liabilities that we may incur. We anticipate that we will need to increase our insurance coverage when we begin clinical trials and if we successfully commercialize any drug candidate. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

***If we or our collaborators are unable to successfully develop and commercialize companion diagnostics for our drug candidates, or experience significant delays in doing so we may not realize the full commercial potential of our drug candidates.***

Because we are focused on precision medicine, in which predictive biomarkers will be used to identify the right patients for our drug candidates, we believe that our success may depend, in part, on the development and commercialization of companion diagnostics. There has been limited success to date industrywide in developing and commercializing these types of companion diagnostics. To be successful, we need to address a number of scientific, technical and logistical challenges. We have not yet initiated development and commercialization of companion diagnostics. We have little experience in the development and commercialization of diagnostics and may not be successful in developing and commercializing appropriate diagnostics to pair with any of our drug candidates that receive marketing approval. Companion diagnostics are subject to regulation by the FDA and similar regulatory authorities outside the United States as medical devices and require separate regulatory approval prior to commercialization. Given our limited experience in developing and commercializing diagnostics, we expect to rely in part or in whole on third parties for their design, manufacture and commercialization. We and our collaborators may encounter difficulties in developing and obtaining approval for the companion diagnostics, including issues relating to selectivity/specificity, analytical validation, reproducibility, or clinical validation. Any delay or failure by us or our collaborators to develop or obtain regulatory approval of the companion diagnostics could delay or prevent approval of our drug candidates. If we, or any third parties that we engage to assist us, are unable to successfully develop and commercialize companion diagnostics for our drug candidates, or experience delays in doing so:

- the development of our drug candidates may be adversely affected if we are unable to appropriately select patients for enrollment in our clinical trials;
- our drug candidates may not receive marketing approval if safe and effective use of a therapeutic drug candidate depends on an *in vitro* diagnostic; and
- we may not realize the full commercial potential of any drug candidates that receive marketing approval if, among other reasons, we are unable to appropriately select patients who are likely to benefit from therapy with our drugs.

As a result, our business would be harmed, possibly materially.

In addition, third party collaborators may encounter production difficulties that could constrain the supply of the companion diagnostics, and both they and we may have difficulties gaining acceptance of the use of the companion diagnostics in the clinical community. If such companion diagnostics fail to gain market acceptance, it would have an adverse effect on our ability to derive revenues from sales of our drug candidates, if approved. In addition, the diagnostic company with whom we contract may decide to discontinue selling or manufacturing the companion diagnostic that we anticipate using in connection with development and commercialization of our drug candidates or our relationship with such diagnostic company may otherwise terminate. We may not be able to enter into arrangements with another diagnostic company to obtain supplies of an alternative diagnostic test for use in connection with the development and commercialization of our drug candidates or do so on commercially reasonable terms, which could adversely affect and/or delay the development or commercialization of our drug candidates.

***Even if we are able to commercialize any drug candidates, such drugs may become subject to unfavorable pricing regulations or third-party coverage and reimbursement policies, which would harm our business.***

The regulations that govern regulatory approvals, pricing and reimbursement for new drugs vary widely from country to country. Some countries require approval of the sale price of a drug before it can be marketed. In many

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

Our ability to commercialize any drug candidates successfully also will depend in part on the extent to which coverage and reimbursement for these drug candidates and related treatments will be available from government authorities, private health insurers and other organizations. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular drugs. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for drugs. We cannot be sure that coverage will be available for any drug candidate that we commercialize and, if coverage is available, the level of reimbursement. Reimbursement may impact the demand for, or the price of, any drug candidate for which we obtain marketing approval. If reimbursement is not available or is available only to limited levels, we may not be able to successfully commercialize any drug candidate for which we obtain marketing approval.

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

***Healthcare legislative reform measures may have a material adverse effect on our business and results of operations.***

In the United States, there have been and continue to be a number of legislative initiatives to contain healthcare costs. For example, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or the Affordable Care Act, was passed, which substantially changes the way health care is financed by both governmental and private insurers, and significantly impacts the U.S. pharmaceutical industry. The Affordable Care Act, among other things, subjects biologic products to potential competition by lower-cost biosimilars, addresses a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected, increases the minimum Medicaid rebates owed by manufacturers under the Medicaid Drug Rebate Program and extends the rebate program to individuals enrolled in Medicaid managed care organizations, establishes annual fees and taxes on manufacturers of certain branded prescription drugs, and a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D.

In addition, other legislative changes have been proposed and adopted in the United States since the Affordable Care Act was enacted. On August 2, 2011, the Budget Control Act of 2011 among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least \$1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby

triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions of Medicare payments to providers of 2% per fiscal year. These reductions went into effect on April 1, 2013 and, due to subsequent legislative amendments to the statute, will remain in effect through 2024 unless additional Congressional action is taken. On January 2, 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, further reduced Medicare payments to several types of providers.

Moreover, payment methodologies may be subject to changes in healthcare legislation and regulatory initiatives. For example, the Middle Class Tax Relief and Job Creation Act of 2012 required that the Centers for Medicare & Medicaid Services, the agency responsible for administering the Medicare program, or CMS, reduce the Medicare clinical laboratory fee schedule by 2% in 2013, which served as a base for 2014 and subsequent years. In addition, effective January 1, 2014, CMS also began bundling the Medicare payments for certain laboratory tests ordered while a patient received services in a hospital outpatient setting. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our drug candidates or companion diagnostics or additional pricing pressures.

***If, in the future, we are unable to establish sales and marketing capabilities or enter into agreements with third parties to sell and market our drug candidates, we may not be successful in commercializing our drug candidates if and when they are approved, and we may not be able to generate any revenue.***

We do not currently have a sales or marketing infrastructure and have limited experience in the sale, marketing or distribution of drugs. To achieve commercial success for any approved drug candidate for which we retain sales and marketing responsibilities, we must build our sales, marketing, managerial, and other non-technical capabilities or make arrangements with third parties to perform these services. In the future, we may choose to build a focused sales and marketing infrastructure to sell, or participate in sales activities with our collaborators for, some of our drug candidates if and when they are approved.

There are risks involved with both establishing our own sales and marketing capabilities and entering into arrangements with third parties to perform these services. For example, recruiting and training a sales force is expensive and time consuming and could delay any drug launch. If the commercial launch of a drug candidate for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

Factors that may inhibit our efforts to commercialize our drug candidates on our own include:

- our inability to recruit and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to physicians or persuade adequate numbers of physicians to prescribe any future drugs;
- the lack of complementary drugs to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

If we enter into arrangements with third parties to perform sales, marketing and distribution services, our drug revenues or the profitability of these drug revenues to us are likely to be lower than if we were to market and sell any drug candidates that we develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell and market our drug candidates or may be unable to do so on terms that are favorable to us. We likely will have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our drug candidates effectively. If we do not establish sales and marketing capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our drug

candidates. Further, our business, results of operations, financial condition and prospects will be materially adversely affected.

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

Although we do not currently have any drugs on the market, once we begin commercializing our drug candidates, we will be subject to additional healthcare statutory and regulatory requirements and enforcement by the federal government and the states and foreign governments in which we conduct our business. Healthcare providers, physicians and third-party payors play a primary role in the recommendation and prescription of any drug candidates for which we obtain marketing approval. Our future arrangements with third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute our drug candidates for which we obtain marketing approval. Restrictions under applicable federal and state healthcare laws and regulations, include the following:

- the federal Anti-Kickback Statute 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 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. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;
- the federal False Claims Act imposes criminal and civil penalties, including through civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, 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. In addition, the government may assert that a claim including items and services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program, or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services; similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;
- the federal physician payment transparency requirements, sometimes referred to as the “Sunshine Act” under the Affordable Care Act require manufacturers of drugs, devices, biologics and medical supplies that are reimbursable under Medicare, Medicaid, or the Children’s Health Insurance Program to report to the Department of Health and Human Services information related to physician payments and other transfers of value and the ownership and investment interests of such physicians and their immediate family members;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009 and its implementing regulations, which also imposes obligations on certain covered entity healthcare providers, health plans, and healthcare clearinghouses as well as their business associates that perform certain services involving the use or disclosure of individually identifiable health information, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information; and
- analogous state laws and regulations, such as state anti-kickback and false claims laws that may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers; and some state laws require



pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring drug manufacturers to report information related to payments to physicians and other health care providers or marketing expenditures, and state 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.

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

***Our future growth may depend, in part, on our ability to penetrate foreign markets, where we would be subject to additional regulatory burdens and other risks and uncertainties.***

Our future profitability may depend, in part, on our ability to commercialize our drug candidates in foreign markets for which we may rely on collaboration with third parties. We are not permitted to market or promote any of our drug candidates before we receive regulatory approval from the applicable regulatory authority in that foreign market, and we may never receive such regulatory approval for any of our drug candidates. To obtain separate regulatory approval in many other countries we must comply with numerous and varying regulatory requirements of such countries regarding safety and efficacy and governing, among other things, clinical trials and commercial sales, pricing and distribution of our drug candidates, and we cannot predict success in these jurisdictions. If we obtain approval of our drug candidates and ultimately commercialize our drug candidates in foreign markets, we would be subject to additional risks and uncertainties, including:

- our customers' ability to obtain reimbursement for our drug candidates in foreign markets;
- our inability to directly control commercial activities because we are relying on third parties;
- the burden of complying with complex and changing foreign regulatory, tax, accounting and legal requirements;
- different medical practices and customs in foreign countries affecting acceptance in the marketplace;
- import or export licensing requirements;
- longer accounts receivable collection times;
- longer lead times for shipping;
- language barriers for technical training;
- reduced protection of intellectual property rights in some foreign countries;
- the existence of additional potentially relevant third-party intellectual property rights;
- foreign currency exchange rate fluctuations; and

·the interpretation of contractual provisions governed by foreign laws in the event of a contract dispute.

Foreign sales of our drug candidates could also be adversely affected by the imposition of governmental controls, political and economic instability, trade restrictions and changes in tariffs.

***Governments outside the United States tend to impose strict price controls, which may adversely affect our revenues, if any.***

In some countries, particularly the countries in Europe, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a drug. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our drug candidate to other available therapies. If reimbursement of our drugs is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be harmed, possibly materially.

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

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

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

#### **Risks Related to Our Dependence on Third Parties**

***We may seek to establish additional collaborations, and, if we are not able to establish them on commercially reasonable terms, we may have to alter our development and commercialization plans.***

Our drug development programs and the potential commercialization of our drug candidates will require substantial additional cash to fund expenses. For some of our drug candidates, we may decide to collaborate with additional pharmaceutical and biotechnology companies for the development and potential commercialization of those drug candidates.

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential market for the subject drug candidate, the costs and complexities of manufacturing and delivering such drug candidate to patients, the potential of competing drugs, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge and industry and market conditions generally. The collaborator may also consider alternative drug candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us for our drug candidate. The terms of any additional collaborations or other arrangements that we may establish may not be favorable to us.

We may also be restricted under existing collaboration agreements from entering into future agreements on certain terms with potential collaborators. Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

We may not be able to negotiate additional collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of the drug candidate for which we are seeking to collaborate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our drug candidates or bring them to market and generate drug revenue.

In addition, our collaboration with Alexion and any future collaborations that we enter into may not be successful. The success of our collaboration arrangements will depend heavily on the efforts and activities of our collaborators. Collaborators generally have significant discretion in determining the efforts and resources that they will apply to these collaborations. Disagreements between parties to a collaboration arrangement regarding clinical development and commercialization matters can lead to delays in the development process or commercializing the applicable drug candidate and, in some cases, termination of the collaboration arrangement. These disagreements can be difficult to resolve if neither of the parties has final decision-making authority. Collaborations with pharmaceutical or biotechnology companies and other third parties often are terminated or allowed to expire by the other party. Any such termination or expiration would adversely affect us financially and could harm our business reputation.

***We expect to rely on third parties to conduct our clinical trials for our drug candidates. If these third parties do not successfully carry out their contractual duties, comply with regulatory requirements or meet expected deadlines, we may not be able to obtain regulatory approval for or commercialize our drug candidates and our business could be substantially harmed.***

We do not have the ability to independently conduct clinical trials. We expect to rely on medical institutions, clinical investigators, contract laboratories and other third parties, such as CROs, to conduct or otherwise support clinical trials for our drug candidates. We expect to rely heavily on these parties for execution of clinical trials for our drug candidates and control only certain aspects of their activities. Nevertheless, we will be responsible for ensuring that each of our clinical trials is conducted in accordance with the applicable protocol, legal and regulatory requirements and scientific standards, and our reliance on CROs will not relieve us of our regulatory responsibilities. For any violations of laws and regulations during the conduct of our clinical trials, we could be subject to warning letters or enforcement action that may include civil penalties up to and including criminal prosecution.

We and our CROs will be required to comply with regulations, including GCPs, for conducting, monitoring, recording and reporting the results of clinical trials to ensure that the data and results are scientifically credible and accurate, and that the trial patients are adequately informed of the potential risks of participating in clinical trials and their rights are protected. These regulations are enforced by the FDA, the Competent Authorities of the Member States of the European Economic Area and comparable foreign regulatory authorities for any drugs in clinical development. The FDA enforces GCP regulations through periodic inspections of clinical trial sponsors, principal investigators and trial sites. If we or our CROs fail to comply with applicable GCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot assure you that, upon inspection, the FDA will determine that any of our future clinical trials will comply with GCPs. In addition, our clinical trials must be conducted with drug candidates produced under cGMPs regulations. Our failure or the failure of our CROs to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process and could also subject us to enforcement action. We also are required to register ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within certain timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions.

Although we intend to design the clinical trials for our drug candidates, CROs will conduct all of the clinical trials. As a result, many important aspects of our development programs, including their conduct and timing, will be outside of our direct control. Our reliance on third parties to conduct future clinical trials will also result in less direct control over the management of data developed through clinical trials than would be the case if we were relying entirely upon our own staff. Communicating with outside parties can also be challenging, potentially leading to mistakes as well as difficulties in coordinating activities. Outside parties may:

- have staffing difficulties;
- fail to comply with contractual obligations;
- experience regulatory compliance issues;
- undergo changes in priorities or become financially distressed; or
- form relationships with other entities, some of which may be our competitors.

These factors may materially adversely affect the willingness or ability of third parties to conduct our clinical trials and may subject us to unexpected cost increases that are beyond our control. If the CROs do not perform clinical trials in a satisfactory manner, breach their obligations to us or fail to comply with regulatory requirements, the development, regulatory approval and commercialization of our drug candidates may be delayed, we may not be able to obtain regulatory approval and commercialize our drug candidates, or our development program materially and irreversibly harmed. If we are unable to rely on clinical data collected by our CROs, we could be required to repeat, extend the duration of, or increase the size of any clinical trials we conduct and this could significantly delay commercialization and require significantly greater expenditures.

If any of our relationships with these third-party CROs terminate, we may not be able to enter into arrangements with alternative CROs. If CROs do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols, regulatory requirements or for other reasons, any clinical trials such CROs are associated with may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for or successfully commercialize our drug candidates. As a result, we believe that our financial results and the commercial prospects for our drug candidates in the subject indication would be harmed, our costs could increase and our ability to generate revenue could be delayed.

***We contract with third parties for the manufacture of our drug candidates for pre-clinical development and expect to continue to do so for clinical testing and commercialization. This reliance on third parties increases the risk that we will not have sufficient quantities of our drug candidates or drugs or such quantities at an acceptable cost, which could delay, prevent or impair our development or commercialization efforts.***

We do not currently own or operate, nor do we have any plans to establish in the future, any manufacturing facilities or personnel. We rely, and expect to continue to rely, on third parties for the manufacture of our drug candidates for pre-clinical development and clinical testing, as well as for the commercial manufacture of our drugs if any of our drug candidates receive marketing approval. This reliance on third parties increases the risk that we will not have sufficient quantities of our drug candidates or drugs or such quantities at an acceptable cost or quality, which could delay, prevent or impair our development or commercialization efforts.

The facilities used by our contract manufacturers to manufacture our drug candidates must be approved by the FDA pursuant to inspections that will be conducted after we submit our marketing applications to the FDA. We do not control the manufacturing process of, and will be completely dependent on, our contract manufacturers for compliance with cGMPs in connection with the manufacture of our drug candidates. If our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or others, they will not be able to secure and/or maintain regulatory approval for their manufacturing facilities. In addition, we have no control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or a comparable foreign regulatory authority does not approve these facilities for the manufacture of our drug candidates or if it withdraws any such approval in the future, we may need to find alternative manufacturing facilities, which would significantly impact our ability to develop, obtain regulatory

approval for or market our drug candidates, if approved. Further, our failure, or the failure of our third party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of drug candidates or drugs, if approved, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect our business and supplies of our drug candidates.

We may be unable to establish any agreements with third-party manufacturers or to do so on acceptable terms. Even if we are able to establish agreements with third party manufacturers, reliance on third party manufacturers entails additional risks, including:

- reliance on the third party for regulatory compliance and quality assurance;
- the possible breach of the manufacturing agreement by the third party;
- the possible misappropriation of our proprietary information, including our trade secrets and know-how; and
- the possible termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us.

Our drug candidates and any drugs that we may develop may compete with other drug candidates and approved drugs for access to manufacturing facilities. There are a limited number of manufacturers that operate under cGMP regulations and that might be capable of manufacturing for us.

Any performance failure on the part of our existing or future manufacturers could delay clinical development or marketing approval. If our current contract manufacturers cannot perform as agreed, we may be required to replace such manufacturers. Although we believe that there are several potential alternative manufacturers who could manufacture our drug candidates, we may incur added costs and delays in identifying and qualifying any such replacement.

Our current and anticipated future dependence upon others for the manufacture of our drug candidates or drugs may adversely affect our future profit margins and our ability to commercialize any drugs that receive marketing approval on a timely and competitive basis.

***The third parties upon whom we rely for the supply of the active pharmaceutical ingredient, drug product and drug substance used in our lead drug candidates are our sole source of supply, and the loss of any of these suppliers could significantly harm our business.***

The active pharmaceutical ingredients, or API, drug product and drug substance used in our lead drug candidates are supplied to us from single-source suppliers. Our ability to successfully develop our drug candidates, and to ultimately supply our commercial drugs in quantities sufficient to meet the market demand, depends in part on our ability to obtain the API, drug product and drug substance for these drugs in accordance with regulatory requirements and in sufficient quantities for commercialization and clinical testing. We do not currently have arrangements in place for a redundant or second-source supply of any such API, drug product or drug substance in the event any of our current suppliers of such API, drug product and drug substance cease their operations for any reason.

For all of our drug candidates, we intend to identify and qualify additional manufacturers to provide such API, drug product and drug substance prior to submission of an NDA to the FDA and/or an MAA to the EMA. We are not certain, however, that our single-source suppliers will be able to meet our demand for their products, either because of the nature of our agreements with those suppliers, our limited experience with those suppliers or our relative importance as a customer to those suppliers. It may be difficult for us to assess their ability to timely meet our demand in the future based on past performance. While our suppliers have generally met our demand for their products on a timely basis in the past, they may subordinate our needs in the future to their other customers.

Establishing additional or replacement suppliers for the API, drug product and drug substance used in our drug candidates, if required, may not be accomplished quickly. If we are able to find a replacement supplier, such replacement supplier would need to be qualified and may require additional regulatory approval, which could result in further delay. While we seek to maintain adequate inventory of the API, drug product and drug substance used in our drug candidates, any interruption or delay in the supply of components or materials, or our inability to obtain such API, drug product and

drug substance from alternate sources at acceptable prices in a timely manner could impede, delay, limit or prevent our development efforts, which could harm our business, results of operations, financial condition and prospects.

### **Risks Related to Intellectual Property**

***If we are unable to adequately protect our proprietary technology or obtain and maintain patent protection for our technology and drugs or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize technology and drugs similar or identical to ours, and our ability to successfully commercialize our technology and drugs may be impaired.***

Our commercial success depends in part on our ability to obtain and maintain proprietary or intellectual property protection in the United States and other countries for our drug candidates, including BLU-285 and BLU-554, and our core technologies, including our novel target discovery engine and our proprietary compound library and other know-how. We seek to protect our proprietary and intellectual property position by, among other methods, filing patent applications in the United States and abroad related to our proprietary technology, inventions and improvements that are important to the development and implementation of our business. We also rely on trade secrets, know-how and continuing technological innovation to develop and maintain our proprietary and intellectual property position.

We own a patent and patent applications that relate to BLU-285 and BLU-554 as composition of matter. We also own applications relating to composition of matter for KIT Exon 17 inhibitors with different compound families, composition of matter for FGFR4 inhibitors with multiple compound families, and composition of matter for inhibitors of the predicted RET resistant mutants, as well as methods of use for these novel compounds. The issued patent directed to BLU-554 composition of matter is expected to expire in 2033, and any patents issuing from our pending patent applications are projected to expire between 2034 and 2036.

As of March 31, 2015, we owned four pending U.S. patent applications, nine pending foreign patent applications and three pending Patent Cooperation Treaty, or PCT, patent applications that relate to our KIT Exon 17 program. Any U.S. or ex-U.S. patents issuing from the pending applications covering BLU-285 will have a statutory expiration date of October 2034. Patent term adjustments or patent term extensions could result in later expiration dates.

As of March 31, 2015, we owned one issued U.S. patent, three pending U.S. patent applications, 32 foreign patent applications corresponding to two of these pending U.S. applications, and two pending PCT patent applications that relate to our FGFR4 program. Each of the U.S. and ex-U.S. patent issuing from the pending applications covering BLU-554 will have a statutory expiration date of July 2033, December 2033, or October 2034. Patent term adjustments or patent term extensions could result in later expiration dates.

As of March 31, 2015, we owned one pending U.S. patent application that relates to our RET program.

The intellectual property portfolio directed to our platform includes patent applications directed to novel gene fusions and the uses of these fusions for detecting and treating conditions implicated with these fusions. As of March 31, 2015, we owned six pending U.S. patent applications and four PCT patent applications related to our platform.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation.

The degree of patent protection we require to successfully commercialize our drug candidates may be unavailable or severely limited in some cases and may not adequately protect our rights or permit us to gain or keep any competitive advantage. We cannot provide any assurances that any of our patents have, or that any of our pending patent applications that mature into issued patents will include, claims with a scope sufficient to protect BLU-285, BLU-554 or our other drug candidates. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States. Furthermore, patents have a limited lifespan. In the United States, the natural expiration of a patent is generally twenty years after it is filed. Various extensions may be available; however, the life of a patent, and the protection it affords, is limited. Given the amount of time required for the development, testing and regulatory review of new drug candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with adequate and continuing patent protection sufficient to exclude others from commercializing drugs similar or identical to our drug candidates, including generic versions of such drugs.

Other parties have developed technologies that may be related or competitive to our own, and such parties may have filed or may file patent applications, or may have received or may receive patents, claiming inventions that may overlap or conflict with those claimed in our own patent applications or issued patents, with respect to either the same methods or formulations or the same subject matter, in either case that we may rely upon to dominate our patent position in the market. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot know with certainty whether we were the first to make the inventions claimed in our owned or licensed patents or pending patent applications, or that we were the first to file for patent protection of such inventions. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights cannot be predicted with any certainty.

In addition, the patent prosecution process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. Further, with respect to most of the pending patent applications covering our drug candidates, prosecution has yet to commence. Patent prosecution is a lengthy process, during which the scope of the claims initially submitted for examination by the U.S. Patent and Trademark Office, or USPTO, have been significantly narrowed by the time they issue, if at all. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Moreover, in some circumstances, we do not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering technology that we license from third parties. Therefore, these patents and applications may not be prosecuted and enforced in a manner consistent with the best interests of our business.

Even if we acquire patent protection that we expect should enable us to maintain such competitive advantage, third parties may challenge the validity, enforceability or scope thereof, which may result in such patents being narrowed, invalidated or held unenforceable. The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and abroad. For example, we may be subject to a third-party submission of prior art to the USPTO challenging the priority of an invention claimed within one of our patents, which submissions may also be made prior to a patent's issuance, precluding the granting of any of our pending patent applications. We may become involved in opposition, derivation, reexamination, *inter partes* review, post-grant review or interference proceedings challenging our patent rights or the patent rights of others from whom we have obtained licenses to such rights. Competitors may claim that they invented the inventions claimed in our issued patents or patent applications prior to us, or may file patent applications before we do. Competitors may also claim that we are infringing on their patents and that we therefore cannot practice our technology as claimed under our patents, if issued. Competitors may also contest our patents, if issued, by showing the patent examiner that the invention was not original, was not novel or was obvious. In litigation, a competitor could claim that our patents, if issued, are not valid for a number of reasons. If a court agrees, we would lose our rights to those challenged patents.

In addition, we may in the future be subject to claims by our former employees or consultants asserting an ownership right in our patents or patent applications, as a result of the work they performed on our behalf. Although we generally require all of our employees, consultants and advisors and any other third parties who have access to our proprietary know-how, information or technology to assign or grant similar rights to their inventions to us, we cannot be certain that we have executed such agreements with all parties who may have contributed to our intellectual property, nor can we be certain that our agreements with such parties will be upheld in the face of a potential challenge, or that they will not be breached, for which we may not have an adequate remedy.

An adverse determination in any such submission or proceeding may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and drugs, without payment to us, or could limit the duration of the patent protection covering our technology and drug candidates. Such challenges may also result in our inability to manufacture or commercialize our drug candidates without infringing third party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future drug candidates.

Even if they are unchallenged, our issued patents and our pending patents, if issued, may not provide us with any meaningful protection or prevent competitors from designing around our patent claims to circumvent our owned or licensed patents by developing similar or alternative technologies or drugs in a non-infringing manner. For example, a third party may develop a competitive drug that provides benefits similar to one or more of our drug candidates but that has a different composition that falls outside the scope of our patent protection. If the patent protection provided by the patents and patent applications we hold or pursue with respect to our drug candidates is not sufficiently broad to impede such competition, our ability to successfully commercialize our drug candidates could be negatively affected, which would harm our business.

***Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could have a material adverse effect on the success of our business.***

Our commercial success depends upon our ability and the ability of our collaborators to develop, manufacture, market and sell our drug candidates and use our proprietary technologies without infringing the proprietary rights and intellectual property of third parties. The biotechnology and pharmaceutical industries are characterized by extensive and frequent litigation regarding patents and other intellectual property rights. We may in the future become party to, or threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to our drug candidates and technology, including interference proceedings before the USPTO. Our competitors or other third parties may assert infringement claims against us, alleging that our drugs are covered by their patents. Given the vast number of patents in our field of technology, we cannot be certain that we do not infringe existing patents or that we will not infringe patents that may be granted in the future. Many companies have filed, and continue to file, patent applications related to kinase inhibitors. Some of these patent applications have already been allowed or issued, and others may issue in the future. 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. Furthermore, because patent applications can take many years to issue and may be confidential for 18 months or more after filing, and because pending patent claims can be revised before issuance, there may be applications now pending which may later result in issued patents that may be infringed by the manufacture, use or sale of our drug candidates. If a patent holder believes our drug or drug candidate infringes on its patent, the patent holder may sue us even if we have received patent protection for our technology. Moreover, we may face patent infringement claims from non-practicing entities that have no relevant drug revenue and against whom our own patent portfolio may thus have no deterrent effect.

If we are found to infringe a third party's intellectual property rights, we could be required to obtain a license from such third party to continue developing and marketing our drug candidates and technology. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain such a license, it could be granted on non-exclusive terms, thereby providing our competitors and other third parties access to the same technologies licensed to us. Without such a license, we could be forced, including by court order, to cease developing and commercializing the infringing technology or drug candidates. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees if we are found to have willfully infringed such third-party patent rights. A finding of infringement could prevent us from commercializing our drug candidates or force us to cease some of our business operations, which could materially harm our business.

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

Competitors and other third parties may infringe, misappropriate or otherwise violate our patents and other intellectual property rights. To counter infringement or unauthorized use, we may be required to file infringement claims. A court may disagree with our allegations, however, and may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the third-party technology in question. Further, such third parties could counterclaim that we infringe their intellectual property or that a patent we have asserted against them is invalid or unenforceable. In patent litigation in the United States, defendant counterclaims challenging the validity, enforceability or scope of asserted patents are commonplace. In addition, third parties may initiate legal proceedings against us to assert such challenges to our intellectual property rights. The outcome of any such proceeding is generally unpredictable. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack



of novelty, obviousness or non-enablement. Patents may be unenforceable if someone connected with prosecution of the patent withheld relevant information from the USPTO or made a misleading statement during prosecution. It is possible that prior art of which we and the patent examiner were unaware during prosecution exists, which could render our patents invalid. Moreover, it is also possible that prior art may exist that we are aware of but do not believe is relevant to our current or future patents, but that could nevertheless be determined to render our patents invalid.

An adverse result in any litigation proceeding could put one or more of our patents at risk of being invalidated or interpreted narrowly. If a defendant were to prevail on a legal assertion of invalidity or unenforceability of our patents covering one of our drug candidates, we would lose at least part, and perhaps all, of the patent protection covering such drug candidate. Competing drugs may also be sold in other countries in which our patent coverage might not exist or be as strong. If we lose a foreign patent lawsuit, alleging our infringement of a competitor's patents, we could be prevented from marketing our drugs in one or more foreign countries. Any of these outcomes would have a materially adverse effect on our business.

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

Litigation or other legal proceedings relating to intellectual property claims, with or without merit, is unpredictable and generally expensive and time consuming and is likely to divert significant resources from our core business, including distracting our technical and management personnel from their normal responsibilities. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities.

We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources and more mature and developed intellectual property portfolios. Accordingly, despite our efforts, we may not be able to prevent third parties from infringing upon or misappropriating or from successfully challenging our intellectual property rights. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have a material adverse effect on our ability to compete in the marketplace.

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

The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. In addition, periodic maintenance fees on issued patents often must be paid to the USPTO and foreign patent agencies over the lifetime of the patent. While an unintentional lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Non-compliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. If we fail to maintain the patents and patent applications covering our drugs or procedures, we may not be able to stop a competitor from marketing drugs that are the same as or similar to our drug candidates, which would have a material adverse effect on our business.

***We may not be able to effectively enforce our intellectual property rights throughout the world.***

Filing, prosecuting and defending patents on our drug candidates in all countries throughout the world would be prohibitively expensive. The requirements for patentability may differ in certain countries, particularly in developing

countries. Moreover, our ability to protect and enforce our intellectual property rights may be adversely affected by unforeseen changes in foreign intellectual property laws. Additionally, the patent laws of some foreign countries do not afford intellectual property protection to the same extent as the laws of the United States. Many companies have encountered significant problems in protecting and defending intellectual property rights in certain foreign jurisdictions. The legal systems of some countries, particularly developing countries, do not favor the enforcement of patents and other intellectual property rights. This could make it difficult for us to stop the infringement of our patents or the misappropriation of our other intellectual property rights. For example, many foreign countries have compulsory licensing laws under which a patent owner must grant licenses to third parties. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own drugs and, further, may export otherwise infringing drugs to territories where we have patent protection, if our ability to enforce our patents to stop infringing activities is inadequate. These drugs may compete with our drug candidates, and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Proceedings to enforce our patent rights in foreign jurisdictions, whether or not successful, could result in substantial costs and divert our efforts and resources from other aspects of our business. Furthermore, while we intend to protect our intellectual property rights in the major markets for our drug candidates, we cannot ensure that we will be able to initiate or maintain similar efforts in all jurisdictions in which we may wish to market our drug candidates. Accordingly, our efforts to protect our intellectual property rights in such countries may be inadequate.

***Changes to the patent law in the United States and other jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our drug candidates.***

As is the case with other biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involve both technological and legal complexity and is therefore costly, time consuming and inherently uncertain. Recent patent reform legislation in the United States and other countries, including the Leahy-Smith America Invents Act, or Leahy-Smith Act, signed into law on September 16, 2011, could increase those uncertainties and costs. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications are prosecuted, redefine prior art and provide more efficient and cost-effective avenues for competitors to challenge the validity of patents. In addition, the Leahy-Smith Act has transformed the U.S. patent system into a “first to file” system. The first-to-file provisions, however, only became effective on March 16, 2013. Accordingly, it is not yet clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation could make it more difficult to obtain patent protection for our inventions and increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could harm our business, results of operations and financial condition.

The U.S. Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. Additionally, there have been recent proposals for additional changes to the patent laws of the United States and other countries that, if adopted, could impact our ability to obtain patent protection for our proprietary technology or our ability to enforce our proprietary technology. Depending on future actions by the U.S. Congress, the U.S. courts, the USPTO and the relevant law-making bodies in other countries, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future.

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

In addition to the protection afforded by patents, we rely upon unpatented trade secret protection, unpatented know-how and continuing technological innovation to develop and maintain our competitive position. With respect to the building of our proprietary compound library, we consider trade secrets and know-how to be our primary intellectual property. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our collaborators, scientific advisors, employees and consultants, and invention assignment agreements

with our consultants and employees. We may not be able to prevent the unauthorized disclosure or use of our technical know-how or other trade secrets by the parties to these agreements, however, despite the existence generally of confidentiality agreements and other contractual restrictions. Monitoring unauthorized uses and disclosures is difficult, and we do not know whether the steps we have taken to protect our proprietary technologies will be effective. If any of the collaborators, scientific advisors, employees and consultants who are parties to these agreements breaches or violates the terms of any of these agreements, we may not have adequate remedies for any such breach or violation, and we could lose our trade secrets as a result. Enforcing a claim that a third party illegally obtained and is using our trade secrets, like patent litigation, is expensive and time consuming, and the outcome is unpredictable. In addition, courts outside the United States are sometimes less willing to protect trade secrets.

Our trade secrets could otherwise become known or be independently discovered by our competitors. Competitors could purchase our drug candidates and attempt to replicate some or all of the competitive advantages we derive from our development efforts, willfully infringe our intellectual property rights, design around our protected technology or develop their own competitive technologies that fall outside of our intellectual property rights. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us. If our trade secrets are not adequately protected so as to protect our market against competitors' drugs, our competitive position could be adversely affected, as could our business.

***We may be subject to damages resulting from claims that we or our employees have wrongfully used or disclosed alleged trade secrets of our competitors or are in breach of non-competition or non-solicitation agreements with our competitors.***

We could in the future be subject to claims that we or our employees have inadvertently or otherwise used or disclosed alleged trade secrets or other proprietary information of former employers or competitors. Although we try to ensure that our employees and consultants do not use the intellectual property, proprietary information, know-how or trade secrets of others in their work for us, we may in the future be subject to claims that we caused an employee to breach the terms of his or her non-competition or non-solicitation agreement, or that we or these individuals have, inadvertently or otherwise, used or disclosed the alleged trade secrets or other proprietary information of a former employer or competitor. Litigation may be necessary to defend against these claims. Even if we are successful in defending against these claims, litigation could result in substantial costs and could be a distraction to management. If our defenses to these claims fail, in addition to requiring us to pay monetary damages, a court could prohibit us from using technologies or features that are essential to our drug candidates, if such technologies or features are found to incorporate or be derived from the trade secrets or other proprietary information of the former employers. An inability to incorporate such technologies or features would have a material adverse effect on our business, and may prevent us from successfully commercializing our drug candidates. In addition, we may lose valuable intellectual property rights or personnel as a result of such claims. Moreover, any such litigation or the threat thereof may adversely affect our ability to hire employees or contract with independent sales representatives. A loss of key personnel or their work product could hamper or prevent our ability to commercialize our drug candidates, which would have an adverse effect on our business, results of operations and financial condition.

#### **Risks Related to Employee Matters, Managing Growth and Other Risks Related to Our Business**

***Our future success depends on our ability to retain key executives and to attract, retain and motivate qualified personnel.***

We are highly dependent on the research and development, clinical and business development expertise of Jeffrey W. Albers, our President and Chief Executive Officer, Anthony L. Boral, our Senior Vice President, Clinical Development, Kyle D. Kovalanka, our Chief Business Officer, and Christoph Lengauer, our Chief Scientific Officer, as well as the other principal members of our management, scientific and clinical team. Although we have entered into employment letter agreements with our executive officers, each of them may terminate their employment with us at any time. We do not maintain "key person" insurance for any of our executives or other employees. In addition, we rely on

consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our growth strategy will be limited.

Recruiting and retaining qualified scientific, clinical, manufacturing and sales and marketing personnel will also be critical to our success. The loss of the services of our executive officers or other key employees could impede the achievement of our research, development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, replacing executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to successfully develop, gain regulatory approval of and commercialize drugs. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these key personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. Failure to succeed in clinical trials may make it more challenging to recruit and retain qualified scientific personnel.

***We will need to develop and expand our company, and we may encounter difficulties in managing this development and expansion, which could disrupt our operations.***

As of March 31, 2015 we had 60 full-time employees, and in connection with becoming a public company, we expect to increase our number of employees and the scope of our operations. To manage our anticipated development and expansion, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Also, our management may need to divert a disproportionate amount of its attention away from its day-to-day activities and devote a substantial amount of time to managing these development activities. Due to our limited resources, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. This may result in weaknesses in our infrastructure, give rise to operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. The physical expansion of our operations may lead to significant costs and may divert financial resources from other projects, such as the development of our drug candidates. If our management is unable to effectively manage our expected development and expansion, our expenses may increase more than expected, our ability to generate or increase our revenue could be reduced and we may not be able to implement our business strategy. Our future financial performance and our ability to commercialize our drug candidates, if approved, and compete effectively will depend, in part, on our ability to effectively manage the future development and expansion of our company.

***Unfavorable global economic conditions could adversely affect our business, financial condition or results of operations.***

Our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets. For example, the global financial crisis caused extreme volatility and disruptions in the capital and credit markets. A severe or prolonged economic downturn, such as the global financial crisis, could result in a variety of risks to our business, including, weakened demand for our drug candidates and our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy could also strain our suppliers, possibly resulting in supply disruption, or cause our customers to delay making payments for our services. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business.

***We or the third parties upon whom we depend may be adversely affected by earthquakes or other natural disasters and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.***

Earthquakes or other natural disasters could severely disrupt our operations, and have a material adverse effect on our business, results of operations, financial condition and prospects. If a natural disaster, power outage or other event

occurred that prevented us from using all or a significant portion of our headquarters, that damaged critical infrastructure, such as the manufacturing facilities of our third-party contract manufacturers, or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible for us to continue our business for a substantial period of time. The disaster recovery and business continuity plans we have in place may prove inadequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which, could have a material adverse effect on our business.

***Our internal computer systems, or those of our third-party CROs or other contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of our drug candidates' development programs.***

Despite the implementation of security measures, our internal computer systems and those of our third-party CROs and other contractors and consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. While we have not experienced any such system failure, accident, or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our programs. For example, the loss of clinical trial data for our drug candidates could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach results in a loss of or damage to our data or applications or other data or applications relating to our technology or drug candidates, or inappropriate disclosure of confidential or proprietary information, we could incur liabilities and the further development of our drug candidates could be delayed.

***Our employees, principal investigators, CROs and consultants may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading.***

We are exposed to the risk that our employees, principal investigators, CROs and consultants may engage in fraudulent conduct or other illegal activity. Misconduct by these parties could include intentional, reckless and/or negligent conduct or disclosure of unauthorized activities to us that violate the regulations of the FDA and other regulatory authorities, including those laws requiring the reporting of true, complete and accurate information to such authorities; healthcare fraud and abuse laws and regulations in the United States and abroad; or laws that require the reporting of financial information or data accurately. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Activities subject to these laws also involve the improper use of information obtained in the course of clinical trials or creating fraudulent data in our pre-clinical studies or clinical trials, which could result in regulatory sanctions and cause serious harm to our reputation. We intend to adopt, prior to the completion of this offering, a code of conduct applicable to all of our employees, but it is not always possible to identify and deter misconduct by employees and other third parties, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. Additionally, we are subject to the risk that a person could allege such fraud or other misconduct, even if none occurred. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of civil, criminal and administrative penalties, damages, monetary fines, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, contractual damages, reputational harm, diminished profits and future earnings, and curtailment of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

***We may acquire businesses or drugs, or form strategic alliances, in the future, and we may not realize the benefits of such acquisitions.***

We may acquire additional businesses or drugs, form strategic alliances or create joint ventures with third parties that we believe will complement or augment our existing business. If we acquire businesses with promising markets or technologies, we may not be able to realize the benefit of acquiring such businesses if we are unable to successfully integrate them with our existing operations and company culture. We may encounter numerous difficulties

in developing, manufacturing and marketing any new drugs resulting from a strategic alliance or acquisition that delay or prevent us from realizing their expected benefits or enhancing our business. We cannot assure you that, following any such acquisition, we will achieve the expected synergies to justify the transaction.

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

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

As a public company, and particularly after we are no longer an “emerging growth company,” we will incur significant legal, accounting and other expenses that we did not incur as a private company. In addition, the Sarbanes-Oxley Act of 2002 and rules subsequently implemented by the Securities and Exchange Commission and NASDAQ have imposed various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs and make some activities more time-consuming and costly. For example, we expect that these rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance.

Pursuant to Section 404 of the Sarbanes-Oxley Act of 2002, or Section 404, we will be required to furnish a report by our management on our internal control over financial reporting, including an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. However, while we remain an emerging growth company, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that neither we nor our independent registered public accounting firm will be able to conclude within the prescribed timeframe that our internal control over financial reporting is effective as required by Section 404. This could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

***Market volatility may affect our stock price and the value of your investment.***

Our IPO was completed on May 5, 2015, where we sold shares of common stock at a price of \$18.00 per share. There has been a public market for our common stock for only a short period of time. Although our common stock is listed on the NASDAQ Global Select Market, an active public market for our common stock may not emerge or be sustained.

In addition, the market price of our common stock may fluctuate significantly in response to a number of factors, most of which we cannot control including among others:

- the success of competitive drugs or technologies;
- results of clinical trials of our drug candidates or those of our competitors;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;

- the level of expenses related to any of our drug candidates or clinical development programs;
- the results of our efforts to discover, develop, acquire or in-license additional drug candidates or drugs;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions; and
- the other factors described in this “Risk Factors” section.

***An active trading market for our common stock may not be sustained, and you may not be able to resell your shares at or above the price you paid.***

Although we have listed our common stock on The NASDAQ Global Select Market, an active trading market for our shares may not be sustained. In the absence of an active trading market for our common stock, investors may not be able to sell their common stock at or above the price at which they acquired their shares or at the time that they would like to sell. An inactive trading market may also impair our ability to raise capital to continue to fund operations by selling shares and may impair our ability to acquire other companies or technologies by using our shares as consideration.

***If securities analysts do not publish research or reports about our business or if they publish negative evaluations of our stock, the price of our stock could decline.***

The trading market for our common stock will rely in part on the research and reports that industry or financial analysts publish about us or our business. We may never obtain research coverage by industry or financial analysts. If no or few analysts commence coverage of us, the trading price of our stock would likely decrease. Even if we do obtain analyst coverage, if one or more of the analysts covering our business downgrade their evaluations of our stock, the price of our stock could decline. If one or more of these analysts cease to cover our stock, we could lose visibility in the market for our stock, which in turn could cause our stock price to decline.

***Our executive officers, directors, principal stockholders and their affiliates exercise significant influence over our company.***

The holdings of our executive officers, directors, principal stockholders and their affiliates, including investment funds affiliated with Third Rock Ventures and entities affiliated with Fidelity Biosciences Corp., or Fidelity, represent beneficial ownership, in the aggregate, of approximately 61% of our outstanding common stock as of March 31, 2015. As a result, these stockholders, if they act together, will be able to influence our management and affairs and the outcome of matters submitted to our stockholders for approval, including the election of directors and any sale, merger, consolidation, or sale of all or substantially all of our assets. In addition, this concentration of ownership might adversely affect the market price of our common stock by:

- delaying, deferring or preventing a change of control of us;
- impeding a merger, consolidation, takeover or other business combination involving us; or
- discouraging a potential acquirer from making a tender offer or otherwise attempting to obtain control of us.

***Anti-takeover provisions in our charter documents and under Delaware law could make an acquisition of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.***

Provisions in our amended and restated certificate of incorporation and amended and restated by-laws may delay or prevent an acquisition of us or a change in our management. These provisions include a classified board of directors, a prohibition on actions by written consent of our stockholders and the ability of our board of directors to issue preferred stock without stockholder approval. In addition, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which limits the ability of stockholders owning in excess of 15% of our outstanding voting stock to merge or combine with us. Although we believe these provisions collectively provide for an opportunity to obtain greater value for stockholders by requiring potential acquirors to negotiate with our board of directors, they would apply even if an offer rejected by our board were considered beneficial by some stockholders. In addition, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors, which is responsible for appointing the members of our management.

***Future sales of our common stock may cause our stock price to decline.***

Sales of a substantial number of shares of our common stock in the public market or the perception that these sales might occur could significantly reduce the market price of our common stock and impair our ability to raise adequate capital through the sale of additional equity securities.

***We have broad discretion in how we use the proceeds from our IPO and may not use these proceeds effectively, which could affect our results of operations and cause our stock price to decline.***

We will have considerable discretion in the application of the net proceeds from our recently completed IPO. As a result, investors will be relying upon management’s judgment with only limited information about our specific intentions for the use of the balance of the net proceeds from the IPO. We may use the net proceeds for purposes that do not yield a significant return or any return at all for our stockholders. In addition, pending their use, we may invest the net proceeds from this the IPO in a manner that does not produce income or that loses value.

***We are an “emerging growth company,” and the reduced disclosure requirements applicable to emerging growth companies may make our common stock less attractive to investors.***

We are an “emerging growth company,” as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. We will remain an emerging growth company until the earlier of (i) the last day of the fiscal year in which we have total annual gross revenues of \$1 billion or more; (ii) the last day of the fiscal year following the fifth anniversary of the date of the completion of our IPO; (iii) the date on which we have issued more than \$1 billion in nonconvertible debt during the previous three years; or (iv) the date on which we are deemed to be a large accelerated filer under the rules of the Securities and Exchange Commission, which means the market value of our common stock that is held by non-affiliates exceeds \$700 million as of the prior June 30th. For so long as we remain an emerging growth company, we are permitted and intend to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include:

- not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002;
- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor’s report providing additional information about the audit and the financial statements;
- providing only two years of audited financial statements in addition to any required unaudited interim financial statements and a correspondingly reduced “Management’s Discussion and Analysis of Financial Condition and Results of Operations” disclosure;



- reduced disclosure obligations regarding executive compensation; and
- exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and shareholder approval of any golden parachute payments not previously approved.

We cannot predict whether investors will find our common stock less attractive if we rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

In addition, the JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. This allows an emerging growth company to delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, we will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

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

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

***Our ability to utilize our net operating loss carryforwards and certain other tax attributes may be limited.***

Under Section 382 of the Internal Revenue Code of 1986, as amended, if a corporation undergoes an “ownership change” (generally defined as a greater than 50% change (by value) in the ownership of its equity over a three year period), the corporation’s ability to use its pre-change net operating loss carryforwards and certain other pre-change tax attributes to offset its post-change income may be limited. We may have experienced such ownership changes in the past, and we may experience ownership changes in the future as a result of this offering or subsequent shifts in our stock ownership, some of which are outside the Company’s control. As of December 31, 2014, we had federal net operating loss carryforwards of approximately \$78.1 million, and our ability to utilize those net operating loss carryforwards could be limited by an “ownership change” as described above, which could result in increased tax liability to the Company.

## **Item 2. Unregistered Sales of Equity Securities and Use of Proceeds**

### *Unregistered Sales of Equity Securities*

During the three months ended March 31, 2015, we issued and sold an aggregate of 10,547 shares of common stock to certain employees and consultants for cash consideration in the aggregate amount of \$19,723 upon the exercise of stock options. These issuances were undertaken in reliance upon the exemption from registration requirements of Rule 701 of the Securities Act as offers and sales of securities under compensatory benefit plans and contracts relating to compensation in compliance with Rule 701.

No underwriters were used in the foregoing transactions.

### *Use of Proceeds from Initial Public Offering of Common Stock*

On May 5, 2015, we closed the sale of 9,367,708 shares of common stock to the public including 1,221,874 shares of common stock issued upon the full exercise by the underwriters of their option to purchase additional shares at a price

of \$18.00 per share, before underwriting discounts. The offer and sale of the shares in our initial public offering was registered under the Securities Act pursuant to a registration statement on Form S-1 (File No. 333-202938), which was filed with the SEC on March 23, 2015 and amended subsequently and declared effective by the SEC on April 29, 2015. Following the sale of the shares in connection with the closing of our initial public offering, the offering terminated. The offering did not terminate before all the securities registered in the registration statements were sold. Goldman, Sachs & Co. and Cowen and Company acted as joint book-running managers for the offering. JMP Securities acted as a co-manager for the offering. Wedbush PacGrow also acted as a co-manager for the offering.

We raised approximately \$154.2 million in net proceeds after deducting underwriting discounts and commissions and estimated offering costs payable by us. None of these expenses consisted of direct or indirect payments made by us to directors, officers or persons owning 10% or more of our common stock or to their associates, or to our affiliates. There has been no material change in the planned use of proceeds from our initial public offering as described in our final prospectus filed with the SEC on April 30, 2015 pursuant to Rule 424(b)(4). We invested the funds received in cash equivalents in accordance with our investment policy.

**Item 6. Exhibits**

The exhibits filed as part of this Quarterly Report on Form 10-Q are set forth on the Exhibit Index, which is incorporated herein by reference.

**SIGNATURES**

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

Blueprint Medicines Corporation

Date: June 11, 2015

By: /s/ Jeffrey W. Albers  
Jeffrey W. Albers  
*President, Chief Executive Officer and Director (Principal Executive Officer)*

Date: June 11, 2015

By: /s/ Kyle D. Kovalanka  
Kyle D. Kovalanka  
*Chief Business Officer (Principal Financial and Accounting Officer)*

**EXHIBIT INDEX**

<b>Exhibit Number</b>	<b>Description of Exhibit</b>
3.2	Form of Fifth Amended and Restated Certificate of Incorporation (incorporated by reference to Exhibit 3.2 to the Company's Registration Statement on Form S-1 (File No. 333-202938) filed on March 23, 2015).
3.3	Form of Amended and Restated Bylaws (incorporated by reference to Exhibit 3.3 to the to the Company's Registration Statement on Form S-1 (File No. 333-202938) filed on March 23, 2015).
4.1	Specimen Common Stock Certificate (incorporated by reference to Exhibit 4.1 to the Company's Registration Statement on Form S-1 (File No. 333-202938), as amended, filed on April 20, 2015).
31.1 *	Certificate of Principal Executive Officer pursuant to Exchange Act rules 13a-14 or 15d-14.
31.2 *	Certificate of Principal Financial Officer pursuant to Exchange Act rules 13a-14 or 15d-14.
32.1 +	Certification of Principal Executive Officer and Principal Financial Officer pursuant to Exchange Act rules 13a-14(b) or 15d-14(b) and 18 U.S.C. Section 1350.
101 *	Interactive Data Files Pursuant to Rule 405 of Regulation S-T: (i) Condensed Balance Sheets as of March 31, 2015 and December 31, 2014, (ii) Condensed Statements of Operations for the three months ended March 31, 2015 and 2014, (iii) Condensed Statements of Cash Flows for the three months ended March 31, 2015 and 2014 and (iv) Notes to Condensed Financial Statements

\* Filed herewith.

+ The certification furnished in Exhibit 32.1 hereto is deemed to be furnished with this Quarterly Report on Form 10-Q and will not be deemed to be "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, except to the extent that the Registrant specifically incorporates it by reference.

## Certifications

I, Jeffrey W. Albers, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Blueprint Medicines Corporation;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:
  - (a). Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - (b). (Paragraph omitted pursuant to SEC Release Nos. 33-8238/34-47986 and 33-8392/34-49313);
  - (c). Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - (d). Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - (a). All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - (b). Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: June 11, 2015

By: /s/ Jeffrey W. Albers  
Jeffrey W. Albers  
President and Chief Executive Officer  
(Principal Executive Officer)

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## Certifications

I, Kyle D. Kovalanka, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Blueprint Medicines Corporation;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:
  - (a). Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - (b). (Paragraph omitted pursuant to SEC Release Nos. 33-8238/34-47986 and 33-8392/34-49313);
  - (c). Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - (d). Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - (a). All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - (b). Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: June 11, 2015

By: /s/ Kyle D. Kovalanka  
Kyle D. Kovalanka  
Chief Business Officer  
(Principal Financial Officer)

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**CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350,  
AS ADOPTED PURSUANT TO  
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with the Quarterly Report on Form 10-Q of Blueprint Medicines Corporation (the "Company") for the period ended March 31, 2015 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), each of the undersigned officers of the Company hereby certifies, pursuant to 18 U.S.C. Section 1350, that to his or her knowledge:

(1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934;  
and

(2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: June 11, 2015

By: /s/ Jeffrey W. Albers  
Jeffrey W. Albers  
President, Chief Executive Officer and Director  
(Principal Executive Officer)

Date: June 11, 2015

By: /s/ Kyle D. Kuvalanka  
Kyle D. Kuvalanka  
Chief Business Officer (Principal Financial and  
Accounting Officer)

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