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

# FORM 10-Q

**(Mark One)**

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

**For the quarterly period ended June 30, 2015**

**OR**

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

**Commission file number 001-37359**

# BLUEPRINT MEDICINES CORPORATION
**(Exact Name of Registrant as Specified in Its Charter)**

| **Delaware** | **26 3632015** |
|---|---|
| **(State or Other Jurisdiction of Incorporation or Organization)** | **(I.R.S. Employer Identification No.)** |
| **215 First St., Cambridge, Massachusetts** | **02142** |
| **(Address of Principal Executive Offices)** | **(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)**

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 | ☐ | Accelerated filer | ☐ |
| Non-accelerated filer | ☒ (Do not check if a smaller reporting company) | Smaller reporting company | ☐ |

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 August 6, 2015: 27,112,120

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

| | June 30, 2015 | December 31, 2014 |
|---|---|---|
| Assets | | |
| Current assets: | | |
| Cash and cash equivalents | $ 193,587 | $ 47,240 |
| Restricted cash | 119 | 119 |
| Unbilled accounts receivable | 1,923 | — |
| Prepaid expenses and other current assets | 5,814 | 915 |
| Total current assets | 201,443 | 48,274 |
| Property and equipment, net | 1,393 | 1,482 |
| Other assets | 36 | 99 |
| Restricted cash | 1,336 | 70 |
| Total assets | $ 204,208 | $ 49,925 |
| Liabilities, convertible preferred stock and stockholders' equity (deficit) | | |
| Current liabilities: | | |
| Accounts payable | 1,611 | 814 |
| Accrued expenses | 4,038 | 3,810 |
| Deferred rent | 58 | 138 |
| Restricted stock liability | 291 | 298 |
| Current portion of deferred revenue | 5,323 | — |
| Current portion of lease incentive obligation | 578 | — |
| Current portion of term loan payable | 2,552 | 1,704 |
| Total current liabilities | 14,451 | 6,764 |
| Deferred rent, net of current portion | 101 | — |
| Restricted stock liability, net of current portion | 19 | 29 |
| Warrant liability | — | 365 |
| Deferred revenue, net of current portion | 10,495 | — |
| Lease incentive obligation, net of current portion | 3,659 | |
| Term loan payable, net of current portion | 5,710 | 7,338 |
| Other long term liabilities | 184 | — |
| Commitments | | |
| Series A convertible preferred stock, $0.001 par value: 0 and 40,150,000 shares authorized at June 30, 2015 and December 31, 2014, respectively; 0 and 40,000,000 shares issued and outstanding at June 30, 2015 and December 31, 2014, respectively | — | 39,958 |
| Series B convertible preferred stock, $0.001 par value: 0 and 20,999,996 shares authorized at June 30,2015 and December 31, 2014, respectively; 0 and 20,916,663 shares issued and outstanding at June 30, 2015 and December 31, 2014, respectively | — | 24,985 |
| Series C convertible preferred stock, $0.001 par value: 0 and 24,154,589 shares authorized at June 30, 2015 and December 31, 2014, respectively; 0 and 24,154,589 shares issued and outstanding at June 30, 2015 and December 31, 2014, respectively | — | 49,868 |
| Stockholders' equity (deficit): | | |
| Preferred stock, $0.001 par value; 5,000,000 shares authorized; no shares issued and outstanding at June 30, 2015 and December 31, 2014 | — | — |
| Common stock, $0.001 par value; 120,000,000 shares authorized; 27,110,402 and 2,218,652 shares issued at June 30, 2015 and December 31, 2014, respectively, and 26,691,946 and 1,626,738 shares outstanding at June 30, 2015 and December 31, 2014, respectively | 27 | 2 |
| Additional paid-in capital | 276,320 | 2,822 |
| Accumulated deficit | (106,758) | (82,206) |
| Total stockholders' equity (deficit) | 169,589 | (79,382) |
| Total liabilities, convertible preferred stock, and stockholders' equity (deficit) | $ 204,208 | $ 49,925 |

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

| | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2015 | 2014 | 2015 | 2014 |
| Collaboration revenue | $ 2,687 | $ — | $ 3,339 | $ — |
| Operating expenses: | | | | |
| Research and development | 11,243 | 6,762 | 20,476 | 12,143 |
| General and administrative | 3,840 | 1,437 | 6,610 | 3,008 |
| Total operating expenses | 15,083 | 8,199 | 27,086 | 15,151 |
| Other income (expense): | | | | |
| Other income (expense), net | (405) | 1 | (441) | 19 |
| Interest expense | (179) | (89) | (364) | (182) |
| Total other income (expense) | (584) | (88) | (805) | (163) |
| Net loss | $ (12,980) | $ (8,287) | $ (24,552) | $ (15,314) |
| Convertible preferred stock dividends | (883) | (1,298) | (3,153) | (2,547) |
| Net loss applicable to common stockholders | $ (13,863) | $ (9,585) | $ (27,705) | $ (17,861) |
| Net loss per share applicable to common stockholders — basic and diluted | $ (0.81) | $ (6.99) | $ (2.94) | $ (13.44) |
| Weighted-average number of common shares used in net loss per share applicable to common stockholders — basic and diluted | 17,093 | 1,371 | 9,430 | 1,329 |

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

| | Six Months Ended June 30, | |
|---|---|---|
| | **2015** | **2014** |
| **Operating activities** | | |
| Net loss | $ (24,552) | $ (15,314) |
| Adjustments to reconcile net loss to net cash used in in operating activities: | | |
| Depreciation and amortization | 411 | 273 |
| Noncash interest expense | 58 | 49 |
| Change in fair value of warrant liability | 445 | (19) |
| Stock-based compensation | 3,014 | 459 |
| Changes in assets and liabilities: | | |
| Unbilled accounts receivable | (1,923) | — |
| Prepaid expenses and other current assets | (639) | (1,193) |
| Other assets | (31) | — |
| Accounts payable | 781 | (590) |
| Accrued expenses | 456 | 1,148 |
| Deferred revenue | 15,818 | — |
| Deferred rent | (3) | (67) |
| Net cash used in operating activities | (6,165) | (15,254) |
| **Investing activities** | | |
| Purchases of property and equipment | (307) | (112) |
| Restricted cash | (1,266) | — |
| Net cash used in investing activities | (1,573) | (112) |
| **Financing activities** | | |
| Proceeds from term loan | — | 2,000 |
| Principal payments on loan payable | (833) | (250) |
| Proceeds from issuance of Series B convertible preferred stock, net of issuance costs | — | 24,986 |
| Proceeds from IPO, net of commissions and underwriting discounts | 156,815 | |
| Payment of offering costs | (1,965) | — |
| Proceeds from issuance of common stock, net of repurchases | 68 | (1) |
| Net cash provided by financing activities | 154,085 | 26,735 |
| Net increase in cash and cash equivalents | 146,347 | 11,369 |
| Cash and cash equivalents at beginning of period | 47,240 | 1,987 |
| Cash and cash equivalents at end of period | $ 193,587 | $ 13,356 |
| **Supplemental cash flow information** | | |
| Cash paid for interest | $ 236 | $ 79 |
| Conversion of convertible preferred stock into common stock | $ 114,808 | $ — |
| Reclassification of warrant liability to additional paid-in-capital | $ 810 | $ — |
| Public offering costs incurred but unpaid at period end | $ 28 | $ — |
| Property and equipment purchases incurred but unpaid at period end | $ 15 | $ — |

**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 drug candidates; the need to develop commercially viable drug candidates; 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 $154.8 million after deducting underwriting discounts and commissions and 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 final prospectus for the IPO 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 June 30, 2015 and the results of its operations for the three and six months ended June 30, 2015 and 2014 and cash flows for the six months ended June 30, 2015 and 2014. Such adjustments are of a normal and recurring nature. The results for the three and six months ended June 30, 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 the quarter ended June 30, 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; 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 granted by the Company to Alexion 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, payment for the achievement of the first milestone under the agreement and payments for certain research and development services. The Company has not received any other milestone payments under the agreement 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 June 30, 2015 and December 31, 2014 (in thousands):

| | June 30, 2015 | December 31, 2014 |
|---|---|---|
| Money market fund | $ 193,587 | $ 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 June 30, 2015, are classified below based on the fair value hierarchy described above:

| Description | June 30, 2015 | Active Markets (Level 1) | Observable Inputs (Level 2) | Unobservable Inputs (Level 3) |
|---|---|---|---|---|
| Money market funds, included in cash equivalents | $ 193,587 | $ 193,587 | $ — | $ — |

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 June 30, 2015 and December 31, 2014, all of the Company's cash equivalents were comprised 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 6). On May 5, 2015, upon completion of the Company's IPO, the warrants to purchase preferred stock converted into warrants to purchase common stock and the Company reclassified the fair value of the warrants as of May 5, 2015 to additional paid-in capital. The following table presents activity in the preferred stock warrant liability during the three and six months ended June 30, 2015 and 2014 (in thousands):

| | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2015 | 2014 | 2015 | 2014 |
| Beginning balance | $ 403 | $ 101 | $ 365 | $ 119 |
| Change in fair value | 407 | (1) | 445 | (19) |
| Reclassification of fair value to additional paid-in capital | (810) | — | (810) | — |
| Ending balance | $ — | $ 100 | $ — | $ 100 |

***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 Company's IPO in May 2015, $2,009 of these costs were recorded in stockholders' equity (deficit) as a reduction of additional paid-in capital generated as a result of the IPO. As of December 31, 2014, the Company recorded $91 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 June 30, 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 June 30, 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, which the Company gained control over on June 15, 2015 and occupancy will commence in October 2015. The lease ends on October 31, 2022. 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 June 30, 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 June 30, 2015 and December 31, 2014.

**4. Accrued Expenses**

Accrued expenses consist of the following (in thousands):

| | June 30, 2015 | December 31, 2014 |
|---|---|---|
| Employee compensation | $ 1,314 | $ 623 |
| External research and development | 1,754 | 2,034 |
| Severance | — | 330 |
| Consulting | 243 | 216 |
| Interest | 37 | 150 |
| Deferred offering costs | 28 | 71 |
| Other | 662 | 386 |
| | $ 4,038 | $ 3,810 |

**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 was 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 $1.2 million, in the aggregate, for the remainder of 2015.

**6. Warrants**

In connection with the 2013 Term Loan (Note 5), 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.

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.

In accordance with ASC 480, the characteristics of these warrants and the rights and privileges of the underlying preferred stock resulted in the classification of these warrants as a liability and were re-measured to the-then current fair value at each balance sheet date. Re-measurement gains or losses were recorded in other income (expense) in the statements of operations. Changes in the fair value of the warrants represented a recurring measurement that was classified within Level 3 of the fair value hierarchy wherein fair value is estimated using significant unobservable inputs.

The Company used the following weighted-average assumptions in its Black-Scholes option pricing model:

| | Series A Warrant | | Series B Warrant | |
|---|---|---|---|---|
| | December 31, 2014 | Issuance | December 31, 2014 | Issuance |
| Fair value of underlying instrument | $ 1.69 | $ 1.00 | $ 1.97 | $ 1.97 |
| Expected volatility | 89.98 % | 80.70 % | 87.38 % | 87.18 % |
| Expected term (in years) | 8.4 | 10.0 | 9.8 | 10.0 |
| Risk-free interest rate | 2.15 % | 2.58 % | 2.24 % | 2.36 % |
| 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 recorded interest expense related to the Series A Warrant and the Series B Warrant of less than $0.1 million in each of the six months ended June 30, 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 the date of the conversion of the warrants, the Company revalued the outstanding warrants using the Black-Scholes option pricing model with the following assumptions:

| | Series A Warrant | Series B Warrant |
|---|---|---|
| | May 5, 2015 | May 5, 2015 |
| Fair value of underlying instrument | $ 20.82 | $ 20.82 |
| Expected volatility | 91.58 % | 87.75 % |
| Expected term (in years) | 8.1 | 9.5 |
| Risk-free interest rate | 2.06 % | 2.19 % |
| Expected dividend yield | — % | — % |

The fair value of the warrants at May 5, 2015 was $0.8 million. The Company recorded other expense of $0.4 million in the statement of operations during the six months ended June 30, 2015 equal to the change in fair value of the warrants from December 31, 2014 to May 5, 2015. The Company reclassified the fair value of the warrants at May 5, 2015, of $0.8 million, to additional paid-in capital.

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.

**7. Stock Awards**

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

On April 8, 2015, the 2015 Stock Option and Incentive Plan (the "Plan") was adopted by the board of directors and approved by the stockholders and became effective upon the completion of the IPO. The Plan replaced the 2011 Stock Option Plan. Any options or awards outstanding under the Company's 2011 Stock Option and Grant Plan remained outstanding and effective. The Plan provides the Company flexibility to use various equity-based incentive and other awards as compensation tools to motivate its workforce. These tools include incentive stock options (ISO), nonstatutory stock options (NSO), 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 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. At June 30, 2015, there were 1,391,097 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 or compensation committee of the board of directors, subject to the provisions of the applicable 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 prior to the IPO, 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. Upon the IPO, the fair value of the common stock on the grant date was based on the closing price of the stock on the date of grant.

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 | (146,129) | 0.31 |
| Repurchased | (22,101) | 0.47 |
| Unvested at June 30, 2015 | 257,049 | 0.53 |

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 six months ended June 30, 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 six months end June 30, 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 $1.3 million of stock-based compensation expense for non-employee performance-based awards in the six months ended June 30, 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 | 1,501,912 | $ 2.02 | 9.42 | $ 7,704 |
| Granted | 652,573 | 10.75 | | |
| Exercised | (41,435) | 1.85 | | |
| Canceled | (14,551) | 2.71 | | |
| Outstanding at June 30, 2015(1) | 2,098,499 | $ 4.74 | 9.16 | $ 45,652 |
| Exercisable at June 30, 2015 | 293,024 | $ 2.61 | 8.82 | $ 6,997 |
| Vested and expected to vest at June 30, 2015(2) | 1,994,592 | $ 4.73 | 9.16 | $ 43,400 |

(1) Includes 162,426 unvested shares of common stock related to early exercises of stock options.

(2) Represents the number of vested options as of June 30, 2015, plus the number of unvested options expected to vest as of June 30, 2015.

(3) Intrinsic value represents the amount by which the fair market value as of June 30, 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 | | Six Months Ended | |
|---|---|---|---|---|
| | June 30, 2015 | June 30, 2014 | June 30, 2015 | June 30, 2014 |
| Risk-free interest rate | 1.53% - 1.92 % | 1.94 % | 1.42% - 1.92 % | 1.91 % |
| Expected dividend yield | — % | — % | — % | — % |
| Expected term (years) | 6 | 6 | 6 | 6 |
| Expected stock price volatility | 82.68 % | 91.36 % | 85.71 % | 91.36 % |

The weighted-average grant date fair value of options granted in the six months ended June 30, 2015 and 2014 was $7.71 and $1.41, respectively. The total intrinsic value of options exercised in the six months ended June 30, 2015 was $0.4 million. There were no options exercised in the six months ended June 30, 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 June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2015 | 2014 | 2015 | 2014 |
| Research and development | $ 897 | $ 124 | $ 1,289 | $ 255 |
| General and administrative | 1,277 | 68 | 1,725 | 204 |
| Total stock-based compensation expense | $ 2,174 | $ 192 | $ 3,014 | $ 459 |

At June 30, 2015, there was $9.6 million of total unrecognized compensation cost related to nonvested stock awards, which is expected to be recognized over a weighted-average period of 2.77 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.

***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. As of June 30, 2015, the first offering under the 2015 Employee Stock Purchase Plan has not occurred.

## 8. 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 as a result of the Company's net loss. 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.

| | June 30, | |
|---|---|---|
| | 2015 | 2014 |
| Convertible preferred stock | — | 11,075,750 |
| Warrants | — | 27,272 |
| Stock options | 2,098,499 | 553,973 |
| Unvested restricted stock | 257,049 | 632,546 |
| Total | 2,355,548 | 12,289,541 |

The weighted average number of common shares used in net loss per share applicable to common stockholders on a basic and diluted basis were 17,092,989 and 1,371,359 for the three months ended June 30, 2015 and 2014, respectively and 9,430,462 and 1,329,302 for the six months ended June 30, 2015 and 2014, respectively.

**9. 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, 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 becomes an affiliate of a biotechnology or pharmaceutical company, and may terminate the agreement 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.

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. Once the milestone is achieved, the Company will recognize revenues from the related milestone payment over the period of performance. In May 2015, the Company achieved the first pre-clinical milestone, which triggered a $1.75 million payment 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.

During the six months ended June 30, 2015, the Company recognized revenue under the Alexion agreement of $3.3 million.

**10. 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 $0.2 million for these services during the six months ended June 30, 2014. The Company did not receive any consulting services from Third Rock Ventures during the three months ended June 30, 2015.

**11. Commitments and Contingencies**

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, which the Company gained control over on June 15, 2015 and occupancy will commence in October 2015. The lease ends on October 31, 2022. The Company has an option to extend the lease for five additional years. The lease has a total committment of $17.8 million over the seven year term. The Company has agreed to pay an initial annual base rent of approximately $2,312,000, which rises periodically until it reaches approximately $2,760,000. The Company is recording rent expense on a straight-line basis through the end of the lease term. The Company has recorded deferred rent on the condensed consolidated balance sheet at June 30, 2015, accordingly. The lease provides the Company with an allowance for leasehold improvements of $4.3 million. The Company accounts for leasehold improvement incentives as a reduction to rent expense ratably over the lease term. The balance from the leasehold improvement incentives is included in lease incentive obligations on the balance sheets. The lease agreement required the Company to pay a security deposit of $1.3 million, which is recorded in restricted cash on the Company’s balance sheet.

## 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 PDGFRα 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, or GIST, 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, or HCC, the most common type of liver cancer. Both drug candidates have demonstrated proof of concept in pre-clinical models. The U.S. Food and Drug Administration, or FDA accepted our Investigational New Drug, or IND, applications to begin Phase 1 clinical trials for BLU-554 for HCC and BLU-285 for GIST. Following these IND approvals, we also filed an IND application for BLU-285 in systemic mastocytosis in August. We plan to initiate our Phase 1 clinical trials for BLU-554 and BLU-285 in 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. RET is a key disease driver in multiple cancers. In pre-clinical studies, our drug candidate BLU6864, induced tumor regression in disease models driven by the primary RET fusion and all predicted secondary on-target resistance mutations. 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. The first pre-clinical milestone was achieved, which triggered a payment of $1.75 million during the three months ended June 30, 2015. Alexion will pay us tiered royalties, ranging from mid-single to low-double digit percentages, 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 the agreement 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 six months ended June 30, 2015, we recognized revenue under the Alexion agreement of $3.3 million.

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 2015. We do not have any drugs approved for sale and have not generated any revenue from drug sales. We have funded our operations primarily through private placements of our convertible preferred stock, debt financing and our initial public offering or IPO. We have raised $115.1 million from the issuance of convertible preferred stock and $10.0 million 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 IPO, at a price to the public of $18.00 per share, resulting in net proceeds of $154.8 million after deducting underwriting discounts and commissions and 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 $24.6 million and $15.3 million for the six months ended June 30, 2015 and 2014, respectively. As of June 30, 2015, we had an accumulated deficit of $106.8 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:

- 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, including BLU6864;
- 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, milestone payment and amounts due to us for research and development services. In the future, revenue may include additional 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, clinical activities and manufacturing on our behalf;
- the cost of consultants;
- the cost of lab supplies and pre-clinical study and clinical trial 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 and six months ended June 30, 2015 and 2014. Pre-development candidate expenses, unallocated costs and internal research and development costs have been classified separately. Internal research and development costs represent personnel expense including stock-based compensation expense.

| | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2015 | 2014 | 2015 | 2014 |
| | (in thousands) | | (in thousands) | |
| BLU-285 external costs | $ 1,271 | $ 820 | $ 2,654 | $ 1,015 |
| BLU-554 external costs | 1,129 | 455 | 2,166 | 663 |
| Pre-development candidate expenses and unallocated costs | 5,902 | 3,363 | 9,571 | 6,451 |
| Internal research and development costs | 2,941 | 2,124 | 6,085 | 4,014 |
| | $ 11,243 | $ 6,762 | $ 20,476 | $ 12,143 |

The substantial increase in external costs associated with BLU-285 and BLU-554 for the three and six months ended June 30, 2015 over the three and six months ended June 30, 2014 was driven by the timing of the nomination of the programs' 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 the re-measurement gain or loss associated with the change in the fair value of the convertible preferred stock warrant liability and 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.

***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 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 granted by us to Alexion 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, payment for the achievement of the first milestone under the agreement and payments for certain research and development services. We have not received any other milestone payments under the agreement 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, or VSOE, of selling price, if available, third-party evidence, or TPE, of selling price if VSOE is not available, or best estimate of selling price, or 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 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 we record 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 June 30, 2015 and 2014***

The following table summarizes our results of operations for the three months ended June 30, 2015 and 2014, together with the changes in those items in dollars and as a percentage:

| | Three Months Ended June 30, | | | |
|---|---|---|---|---|
| | 2015 | 2014 | Dollar Change | % Change |
| | (in thousands) | | | |
| Collaboration revenue | $ 2,687 | $ — | $ 2,687 | 100 % |
| Operating expenses: | | | | |
| Research and development | 11,243 | 6,762 | 4,481 | 66 |
| General and administrative | 3,840 | 1,437 | 2,403 | 167 |
| Total operating expenses | 15,083 | 8,199 | 6,884 | 84 |
| Other income (expense): | | | | |
| Other income (expense), net | (405) | 1 | (406) | (406) |
| Interest expense | (179) | (89) | (90) | (101) |
| Total other income (expense) | (584) | (88) | (496) | (564) |
| Net loss | $ (12,980) | $ (8,287) | $ (4,693) | (57)% |

*Collaboration revenue*

Collaboration revenue was $2.7 million for the three months ended June 30, 2015 under our agreement with Alexion. We did not record any collaboration revenue during the three months ended June 30, 2014.

*Research and Development Expense*

Research and development expense increased by $4.4 million from $6.8 million for the three months ended June 30, 2014 to $11.2 million for the three months ended June 30, 2015, an increase of 66%. The increase in research and development expense was primarily attributable to the following:

- approximately $1.8 million in personnel expense primarily due to (1) a 17% increase in headcount, largely driven by growth in the non-clinical and clinical organizations as our programs advance into clinical trials and (2) higher stock-based compensation expense;
- approximately $1.2 million in external clinical activities as we advance our two lead program clinical trials; and
- approximately $1.3 million as we continue to build our platform and advance our discovery pipeline forward.

*General and Administrative Expense*

General and administrative expense increased by $2.4 million from $1.4 million for the three months ended June 30, 2014 to $3.8 million for the three months ended June 30, 2015, an increase of 167%. The increase in general and administrative expense was primarily attributable to the following:

- approximately $1.9 million in increased personnel costs primarily due to an increase in stock-based compensation expenses as well as an increase of 86% in business personnel headcount to support our overall growth as a publicly traded company; and
- approximately $0.4 million increase in professional fees including external legal fees, insurance premiums, corporate communications and public relations costs.

*Other Income (Expense), Net*

Other expense increased by $0.4 million to $0.4 million for the three months ended June 30, 2015 from $0.001 million of income for the three months ended June 30, 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 June 30, 2015 from $0.1 million for the three months ended June 30, 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 June 30, 2015.

***Comparison of Six Months Ended June 30, 2015 and 2014***

The following table summarizes our results of operations for the six months ended June 30, 2015 and 2014, together with the changes in those items in dollars and as a percentage:

| | Six Months Ended June 30, | | | |
|---|---|---|---|---|
| | 2015 | 2014 | Dollar Change | % Change |
| | (in thousands) | | | |
| Collaboration revenue | $ 3,339 | $ — | $ 3,339 | 100 % |
| Operating expenses: | | | | |
| Research and development | 20,476 | 12,143 | 8,333 | 69 |
| General and administrative | 6,610 | 3,008 | 3,602 | 120 |
| Total operating expenses | 27,086 | 15,151 | 11,935 | 79 |
| Other income (expense): | | | | |
| Other income (expense), net | (441) | 19 | (460) | (2,421) |
| Interest expense | (364) | (182) | (182) | (100) |
| Total other income (expense) | (805) | (163) | (642) | (394) |
| Net loss | $ (24,552) | $ (15,314) | $ (9,238) | (60)% |

*Collaboration revenue*

Collaboration revenue was $3.3 million for the six months ended June 30, 2015 under our agreement with Alexion. We did not record any collaboration revenue during the six months ended June 30, 2014.

*Research and Development Expense*

Research and development expense increased $8.3 million from $12.1 million for the six months ended June 30, 2014 to $20.5 million for the six months ended June 30, 2015, an increase of 69%. The increase in research and development expense was primarily attributable to the following:

- approximately $3.2 million in increased personnel costs primarily due to an increase of 20% 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 $1.9 million in clinical activities as we advance our two lead programs into clinical trials;
- approximately $1.8 million as we continue to build our platform and advance our discovery pipeline forward; and
- approximately $1.2 million for external IND-enabling pre-clinical and toxicology studies as well as manufacturing activities for our two lead programs.

We expect that our research and development expense will increase in future periods as we expand our operations and incur additional costs in connection with our clinical trials. These increases will likely include the costs related to the implementation and expansion of clinical trial sites and related patient enrollment, monitoring, and program management expenses.

*General and Administrative Expense*

General and administrative expense increased by $3.6 million from $3.0 million for the six months ended June 30, 2014 to $6.6 million for the six months ended June 30, 2015, an increase of 120%. The increase in general and administrative expense was primarily attributable to the following:

- approximately $2.5 million in increased personnel costs primarily due to an increase in stock-based compensation expense and an increase of 68% in business personnel headcount to support our overall growth as a publicly traded company; and
- approximately $0.7 million in increase in professional fees including external legal fees, insurance premiums, corporate communications and public relations costs.

*Other Income (Expense), Net*

Other expense increased by $0.4 million to $0.4 million for the six months ended June 30, 2015 from less than $0.02 million of income for the six months ended June 30, 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 $0.2 million to $0.4 million for the six months ended June 30, 2015 from $0.2 million for the six months ended June 30, 2014. The increase in interest expense was primarily related to a higher outstanding principal balance under the Loan and Security Agreement for the six months ended June 30, 2015.

## Liquidity and Capital Resources

***Sources of Liquidity***

Through June 30, 2015, we financed our operations primarily private placements of our convertible preferred stock, debt financing and our initial public offering or IPO. We have raised $115.1 million from the issuance of convertible preferred sotck and $10.0 million 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 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 $154.8 million after deducting underwriting discounts and commissions and offering costs payable by us.

As of June 30, 2015, we had cash and cash equivalents of $193.6 million.

*Cash Flows*

The following table provides information regarding our cash flows for the six months ended June 30, 2015 and 2014:

| | Six Months Ended June 30, | |
|---|---|---|
| **(in thousands)** | **2015** | **2014** |
| Net cash used in operating activities | $ (6,165) | $ (15,254) |
| Net cash used in investing activities | (1,573) | (112) |
| Net cash provided by financing activities | 154,085 | 26,735 |
| Net increase in cash and cash equivalents | $ 146,347 | $ 11,369 |

Net cash used in operating activities was $6.1 million during the six months ended June 30, 2015 compared to net cash used in operating activities of $15.3 million during the six months ended June 30, 2014. The decrease in cash used in operating activities was primarily due to the receipt of the upfront payment from Alexion, partially offset by an increase in net loss of $9.2 million for the six months ended June 30, 2015 as compared to the six months ended June 30, 2014.

Net cash used in investing activities was $1.6 million during the six months ended June 30, 2015 compared to net cash used in investing activities of $0.1 million during the six months ended June 30, 2014. Net cash used in investing activities for the six months ended June 30, 2015 consisted of a security deposit payment for our new office lease agreement as well as purchases of property and equipment. Net cash used in investing activities for the six months ended June 30, 2014 consisted of purchases of property and equipment.

Net cash provided by financing activities was $154.1 million during the six months ended June 30, 2015 compared to net cash provided by financing activities of $26.7 million during the six months ended June 30, 2014. Net cash provided by financing activities for the six months ended June, 2015 was primarily the result of $154.8 million in net proceeds from the IPO after deducting underwriting discounts, commissions and offering costs payable by us. The cash provided by financing activities for the six months ended June 30, 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 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. Based on the composition of our investment portfolio which includes money market funds that invest in U.S. Treasury obligations, the current exposure is immaterial.

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 June 30, 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 six months ended June 30, 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 June 30, 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.. The risk factors set forth below are the only risk factors that have been materially updated from those included in the final prospectus for our IPO, which was filed with the Securities and Exchange Commission on April 30, 2015, or the Prospectus. In addition to considering the risk factors set forth below you should also consider the risk factors included in the Prospectus, 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 these risk factors 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 financed our operations primarily through private placements of our convertible preferred stock, debt financing and our initial public offering or IPO. Through June 30, 2015, we raised an aggregate of $293.9 million of gross proceeds from such transactions. We have raised $115.1 million from the issuance of convertible preferred stock and $10.0 million 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 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 gross proceeds of $168.6 million before deducting underwriting discounts and commissions and offering costs payable by us. As of June 30, 2015, our cash and cash equivalents and investments were $193.6 million. We have incurred net losses in each year since our inception, and we had an accumulated deficit of $106.8 million as of June 30, 2015. Our net losses were $24.6 million and $15.3 million for the six months ended June 30, 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.

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

***We are very early in our development efforts with only two drug candidates scheduled to commence clinical development in 2015. If we are unable to 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 with ontly two drug candidates. BLU-285 and BLU-554, scheduled to commence clinical development in 2015. All of our other drug candidates are currently in pre-clinical development. 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 lead drug candidates will require additional clinical development, management of 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 lead drug candidates and other drug candidates will depend on several factors, including the following:

- successful completion of pre-clinical studies;
- 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.

***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.***

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 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. 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.

## 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 June 30, 2015, we owned four pending U.S. patent applications, eight 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 June 30, 2015, we owned one issued U.S. patent, four pending U.S. patent applications, 30 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 June 30, 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 June 30, 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.

## 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, 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. For example, in August 2015, Kyle D. Kuvalanka announced that he is resigning from his current position as our Chief Business Officer in September 2015. 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.

We expect to continue hiring qualified development personnel. Recruiting and retaining qualified scientific, clinical, manufacturing and sales and marketing personnel will 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 key employees and executive officers such as Mr. Kuvalanka 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.

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

*Issuer Purchases of Equity Securities*

We presently have no publicly announced share repurchase plan or program. All repurchased shares of common stock described in the following table were initially issued as equity incentive awards to employees, directors or consultants in the form of restricted stock or upon the exercise of early-exercisable but unvested stock options. All repurchases were made upon forfeiture of shares of common stock by the recipient of such equity incentive awards in connection with the termination of employment or other service relationship with us. Pursuant to the award agreements governing such grants, the repurchase price for all shares was equal to the price per share initially paid by the recipient. The following table provides information relating to our repurchase of shares of our common stock in the second quarter of 2015.

| Period | Total Number of Shares Purchased | Average Price Paid per Share ($) | Total Number of Shares Purchased as Part of Publicly Announced Plans or Programs | Maximum Number (or Approximate Dollar Value) of Shares that May Yet be Purchased Under the Plans or Programs |
|---|---|---|---|---|
| April 1 - April 30 | — | $ — | — | — |
| May 1 - May 31 | 2,197 | 0.06 | — | — |
| June 1 - June 30 | 4,470 | 0.06 | — | — |
| **Total** | 6,667 | $ 0.06 | — | — |

*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 $154.8 million in net proceeds after deducting underwriting discounts and commissions and 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: August 10, 2015

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

Date: August 10, 2015

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

## EXHIBIT INDEX

| Exhibit Number | Description of Exhibit |
|---|---|
| 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 June 30, 2015 and December 31, 2014, (ii) Condensed Statements of Operations for the three and six months ended June 30, 2015 and 2014, (iii) Condensed Statements of Cash Flows for the six months ended June 30, 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.

**Exhibit 31.1**

**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: August 10, 2015

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

**Exhibit 31.2**

**Certifications**

I, Kyle D. Kuvalanka, 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: August 10, 2015

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

**Exhibit 32.1**

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

In connection with the Quarterly Report on Form 10-Q of Blueprint Medicines Corporation (the "Company") for the period ended June 30, 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: August 10, 2015

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

Date: August 10, 2015

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