

BELLICUM PHARMACEUTICALS, INC

Form 424B4

December 18, 2014

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Filed pursuant to Rule 424(b)(4)
Registration Nos. 333-200328 and 333-201031

PROSPECTUS

7,350,000 Shares

Common Stock

Bellicum Pharmaceuticals, Inc. is offering 7,350,000 shares of its common stock. This is our initial public offering and no public market currently exists for our shares. The initial public offering price of our common stock is \$19.00 per share.

Our common stock has been approved for listing on The NASDAQ Global Market under the symbol BLCM.

We are an emerging growth company as that term is used in the Jumpstart Our Business Startups Act of 2012 and, as such, have elected to comply with certain reduced public company reporting requirements for this prospectus and future filings.

Investing in our common stock involves risks. See Risk Factors beginning on page 12.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities, or determined if this prospectus is truthful or complete. Any representation to the contrary is a criminal offense.

	PER SHARE	TOTAL
Public Offering Price	\$ 19.00	\$ 139,650,000
Underwriting Discounts and Commissions ⁽¹⁾	\$ 1.33	\$ 9,775,500
Proceeds to Bellicum Pharmaceuticals, Inc. (before expenses)	\$ 17.67	\$ 129,874,500

(1) We have agreed to reimburse the underwriters for certain expenses. See Underwriting. Certain of our existing stockholders and their affiliated entities have indicated an interest in purchasing up to approximately \$50.0 million of shares of our common stock in this offering at the public offering price. However, because indications of interest are not binding agreements or commitments to purchase, these entities may determine to purchase more or fewer shares than they have indicated or not to purchase any shares in this offering.

We have granted the underwriters an option for a period of 30 days to purchase up to an additional 1,102,500 shares of common stock. If the underwriters exercise the option in full, the total underwriting discounts and commissions payable by us will be \$11,241,825, and the total proceeds to us, before expenses will be \$149,355,675.

The underwriters expect to deliver the shares of common stock to purchasers on or about December 23, 2014.

Joint Book-Running Managers

Jefferies

Citigroup
Co-Manager

Piper Jaffray

Trout Capital

Prospectus dated December 17, 2014

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Neither we nor any of the underwriters has authorized anyone to provide you with information different from, or in addition to, that contained in this prospectus or any free writing prospectus prepared by or on behalf of us or to which we may have referred you in connection with this offering. We take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may give you. Neither we nor any of the underwriters is making an offer to sell or seeking offers to buy these securities in any jurisdiction where or to any person to whom the offer or sale is not permitted. The information in this prospectus is accurate only as of the date on the front cover of this prospectus, regardless of the time of delivery of this prospectus or of any sale of shares of our common stock and the information in any free writing prospectus that we may provide you in connection with this offering is accurate only as of the date of that free writing prospectus. Our business, financial condition, results of operations and future growth prospects may have changed since those dates.

Through and including January 11, 2015 (25 days after the date of this prospectus), all dealers that effect transactions in these securities, whether or not participating in this offering, may be required to deliver a prospectus. This delivery is in addition to a dealer's obligation to deliver a prospectus when acting as an underwriter and with respect to their unsold allotments or subscriptions.

This prospectus includes statistical and other industry and market data that we obtained from industry publications and research, surveys and studies conducted by third parties. Industry publications and third-party research, surveys and studies generally indicate that their information has been obtained from sources believed to be reliable, although they do not guarantee the accuracy or completeness of such information.

We have obtained registered trademarks for Bellicum[®], CaspaCIDE[®] and DeCIDE[®] based on an intent to use in the United States. We are currently prosecuting registrations for the GoCAR-T and GOCART marks. This prospectus contains references to our trademarks and to trademarks belonging to other entities. Solely for convenience, trademarks and trade names referred to in this prospectus, including logos, artwork and other visual displays, may appear without the [®] or TM symbols, but such references are not intended to indicate, in any way, that we will not assert, to the fullest extent under applicable law, our rights or the rights of the applicable licensor to these trademarks and trade names. We do not intend our use or display of other companies' trade names or trademarks to imply a relationship with, or endorsement or sponsorship of us by, any other companies.

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PROSPECTUS SUMMARY

This summary highlights information contained in other parts of this prospectus. Because it is only a summary, it does not contain all of the information that you should consider before investing in shares of our common stock and it is qualified in its entirety by, and should be read in conjunction with, the more detailed information appearing elsewhere in this prospectus. You should read the entire prospectus carefully, especially Risk Factors beginning on page 12 and our financial statements and the related notes, before deciding to buy shares of our common stock.

Unless the context requires otherwise, references in this prospectus to we, us and our refer to Bellicum Pharmaceuticals, Inc.

Overview

We are a clinical stage biopharmaceutical company focused on discovering and developing novel cellular immunotherapies for various forms of cancer, including both hematological cancers and solid tumors, as well as orphan inherited blood disorders. Cellular immunotherapy has the potential to transform medicine by harnessing immune cells, principally T cells, to attack and eliminate harmful diseased cells in the body. Unlike traditional small molecule and biologic therapies which are predictably metabolized and eliminated from the body, cellular immunotherapies are unpredictable and uncontrollable. We are using our proprietary Chemical Induction of Dimerization, or CID, technology platform to engineer and then control components of the immune system in real time. By incorporating our CID platform, our product candidates may offer better safety and efficacy outcomes than are seen with current cellular immunotherapies.

Our lead clinical product candidate, BPX-501, is an adjunct T-cell therapy administered after allogeneic hematopoietic stem cell transplantation, or HSCT, and is currently being evaluated in multiple Phase 1/2 clinical trials. Our next clinical product candidate, BPX-201, is a dendritic cell cancer vaccine in a Phase 1 clinical trial for the treatment of metastatic castrate-resistant prostate cancer, or mCRPC, targeting the prostate-specific membrane antigen, or PSMA. Dendritic cells are specialized cells that are key regulators of the immune system that process and present antigens on the cell surface to T cells in order to activate the T cells. We are also focused on developing next-generation chimeric antigen receptor, or CAR, T-cell therapies and T-cell receptor, or TCR, therapies and are planning to advance several product candidates into human clinical trials, including: (1) BPX-401, a CAR-T product candidate for hematological cancers that express the CD19 antigen, (2) BPX-601, a CAR-T product candidate for solid tumors overexpressing the prostate stem cell antigen, or PSCA, and (3) BPX-701, a TCR product candidate for solid tumors expressing the preferentially-expressed antigen in melanoma, or PRAME.

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Our product candidate pipeline is set forth below:

Our Proprietary CID Technology Platform

We are developing next-generation product candidates in some of the most important areas of cellular immunotherapy, including HSCT, CAR T cell therapy, and dendritic cell vaccines. HSCT, also known as bone marrow transplantation, has for decades been curative for many patients with hematological cancers or orphan inherited blood disorders. However, application of HSCT is limited by graft-versus-host-disease, or GvHD, a condition in which the transplanted immune cells recognize the host cells as foreign and attack them. Since the transplanted cells can persist indefinitely, GvHD does not resolve by itself and is a major cause of transplant-related morbidity and mortality. CAR T cell therapy is an innovative approach in which a patient's T cells are genetically modified to carry CARs, which redirect the T cells against cancer cells. While high objective response rates have been reported in some hematological malignancies, serious and sometimes fatal toxicities have arisen in patients treated with CAR T cell therapies. These toxicities include instances in which the CAR T cells have caused high levels of cytokines due to over-activation, referred to as cytokine release syndrome, frequent transient neurologic toxicities and cases in which they have attacked healthy organs instead of the targeted tumor, leading to death. In solid tumors, where the behavior of CAR T cells is particularly unpredictable and results have been inconsistent, researchers are developing enhanced CAR T cell approaches called armored CARs that raise even greater safety concerns. Lastly, despite the integral role that dendritic cells play in the immune system, they are difficult to activate appropriately and as a result their use has delivered only modest therapeutic benefit.

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Our proprietary CID technology is designed to address these challenges. Events inside a cell are controlled by cascades of specialized signaling proteins. CID consists of molecular switches, modified forms of these signaling proteins, which are triggered inside the patient by infusion of a small molecule, rimiducid (AP1903), instead of by natural upstream signals. We include these molecular switches in the appropriate immune cells and deliver the cells to the patient in the manner of conventional cellular immunotherapy. We have developed two such switches: a safety switch designed to initiate programmed cell death, or apoptosis, of the immunotherapy cells, and an activation switch designed to stimulate activation and in some cases proliferation of the immunotherapy cells. Each of our technologies incorporates one of these switches, for enhanced, real-time control of safety and efficacy:

- n ***CaspaCIDE*** is our safety switch, incorporated into our HSCT and TCR product candidates, where it is inactive unless the patient experiences a serious side effect. In that event, rimiducid is administered to fully or partially eliminate the cells, with the goal of terminating or attenuating the therapy and resolving the serious side effect.
- n ***CIDeCAR*** consists of CAR T cells modified to include our CaspaCIDE safety switch and in which the CAR incorporates the signaling domains of two proteins, MyD88 and CD40. Together, these form our proprietary dual co-stimulatory domain, MC, which is designed to activate T cells in the presence of cancer cells more potently than co-stimulatory molecules CD28 and 4-1BB, which are used in current CAR T cell therapy. Incorporation of CaspaCIDE in a CIDeCAR product candidate is intended to allow the enhanced potency of MC co-stimulation to be deployed safely in patients.
- n ***GoCAR-T*** consists of CAR T cells that are modified to include the proprietary dual co-stimulatory domain, MC. In contrast to CIDeCAR, MC is structured in GoCAR-T as a molecular switch, separate from the chimeric antigen receptor, which itself contains no co-stimulatory domains. GoCAR-T is designed to allow control of the activation and proliferation of the CAR T cells through the scheduled administration of a course of rimiducid infusions that may continue until the desired patient outcome is achieved. In the event of emergence of side effects, the level of activation of the GoCAR-T cells is designed to be attenuated by reducing the rimiducid administration schedule.
- n ***DeCIDE*** consists of dendritic cells that are modified to include the same MC switch used in GoCAR-T. Upon exposure to rimiducid, dendritic cells containing DeCIDE become highly activated in a process that is less susceptible to being turned off by the immune system's natural inhibitory processes. By administering rimiducid after the patient has been vaccinated and the dendritic cells have had time to migrate to the draining lymph nodes, our DeCIDE product candidates are designed to be activated in a potent and long-lasting manner.

By incorporating our novel switch technologies, we are developing product candidates with the potential to elicit positive clinical outcomes and ultimately change the treatment paradigm in various areas of cellular immunotherapy. Our clinical product candidates, each of which is a combination product of genetically modified immune cells and rimiducid, are described below.

n

BPX-501. We are developing a CaspaCIDE product candidate, BPX-501, as an adjunct T-cell therapy administered after allogeneic HSCT, using donor stem cells. In a typical allogeneic HSCT procedure, a patient receives a full complement of immune cells including both donor stem cells and donor T cells. T cells in the transplant often cause serious and potentially fatal side effects, such as GvHD. BPX-501 is designed to decrease the risk of including T cells with the transplant by enabling the elimination of donor T cells through the triggering of the CaspaCIDE safety switch upon emergence of GvHD. In a 10-patient Phase 1 clinical trial with CaspaCIDE modified T cells, conducted by an academic collaborator, four patients developed GvHD after donor T-cell infusion. A single dose of rimiducid rapidly eliminated over 90% of the modified T cells and resolved GvHD in all four patients without recurrence of GvHD. These findings have been replicated in preliminary data from three patients in a second clinical trial of CaspaCIDE-modified T cells. BPX-501 is currently being evaluated in multiple Phase 1/2 clinical trials in the United States and Europe, with the first top-line data expected in the second half of 2015.

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n **BPX-201.** We are developing a DeCIDE product candidate, BPX-201, as a dendritic cell cancer vaccine made from the patient's own white blood cells, designed to treat mCRPC. It targets the prostate specific membrane antigen, or PSMA, and uses our DeCIDE activation switch technology. BPX-201 is currently being evaluated in an 18-patient Phase 1 clinical trial for mCRPC. We are evaluating opportunities for BPX-201 in combination with other cancer immunotherapies, such as checkpoint inhibitors, which are antibodies designed to block certain inhibitory receptors on the surface of T cells, and thus potentiate the T cells' ability to promote an immune response against cancer. We believe that the increased numbers of PSMA-specific T cells migrating to deposits of prostate cancer in the body that BPX-201 is designed to generate may serve as a substrate for checkpoint inhibitors, resulting in a synergistic, more potent anti-cancer immune response. In addition, our preclinical product candidates are designed to overcome the current limitations of CAR-T and TCR therapies and include the following:

- n **BPX-401.** We are developing a CIDE CAR product candidate, BPX-401, as a next-generation CAR T cell therapy for hematological cancers that express the CD19 antigen. CD19 is an antigen expressed in many hematological cancers, including acute lymphocytic leukemia, or ALL, chronic lymphocytic leukemia, or CLL, and certain non-Hodgkin's lymphomas. We believe that, while the activity of CAR T cell therapy has been demonstrated in early-stage clinical trials by third party researchers in these indications, safety issues, such as cytokine release syndrome, a systemic inflammatory response that is produced by elevated levels of cytokines that are associated with T-cell activation and proliferation, remain a major concern, which may be addressed by BPX-401.
- n **BPX-601.** We are developing a GoCAR-T product candidate, BPX-601, for solid tumors overexpressing PSCA, such as some prostate, pancreatic, bladder, esophageal and gastric cancers. We have obtained positive proof-of-principle data in an animal pancreatic tumor model, which we believe validate BPX-601's activity and rimiducid's ability to modulate therapeutic effect.
- n **BPX-701.** We are developing a CaspaCIDE TCR product candidate, BPX-701, in collaboration with Leiden University Medical Center, initially for the treatment of PRAME-expressing melanoma, sarcomas and neuroblastoma. Based on *in vitro* studies, BPX-701 has demonstrated strong affinity to panels of cancer cells presenting PRAME peptides and low affinity to non-tumor cells. In other *in vitro* studies, rimiducid administration has shown the ability to eliminate BPX-701 cells.

We expect to file investigational new drug applications, or INDs, for BPX-701 in the second half of 2015 and for BPX-401 and BPX-601 in 2016. Our IND-enabling activities for each of these preclinical product candidates include manufacturing key components and developing a robust process to produce cell products that comply with regulations of the U.S. Food and Drug Administration, or FDA, and other regulatory agencies. We have developed an efficient and scalable process to manufacture genetically modified T cells of high quality and purity. This process is being implemented by our third-party contract manufacturers to produce BPX-501 for our clinical trials. We expect to leverage our resources, capabilities and expertise for the manufacture of our CAR-T and TCR product candidates.

Strategy

Our goal is to become a leading innovator in the field of cellular immunotherapy by maximizing the inherent potential of this therapeutic modality and developing medicines with a differentiated combination of safety and efficacy. The key elements of our strategy to achieve this goal are as follows:

- n ***Pursue a broad development strategy that will maximize the market potential of BPX-501.*** We believe that BPX-501 will enable physicians to maximize the benefits of adjunct T-cell therapy for allogeneic HSCT, such as immune system recovery, prevention or treatment of relapse of underlying disease and improvement in stem cell engraftment, while mitigating safety issues associated with the therapy. Based on these attributes, BPX-501 may serve an integral role in the treatment paradigm for allogeneic HSCT in various diseases and increase the overall patient eligibility for the procedure. In

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order to make BPX-501 accessible to a broad group of patients and maximize the market potential of this product candidate, we are conducting multiple Phase 1/2 clinical trials that include U.S. and European protocols, adult and pediatric patients and different indications and usage of BPX-501. We expect to report data from these clinical trials and discuss registration trial design at an end-of-Phase 2 meeting with the FDA and European regulatory authorities in the first half of 2016.

- n ***Focus on developing proprietary CAR-T and TCR product candidates with an improved safety and efficacy profile.*** We intend to build a robust clinical pipeline of our own novel CAR-T and TCR product candidates, which incorporate our proprietary switch technologies, CIDE CAR, GoCAR-T and CaspaCIDE, and focus on indications in which current CAR-T and TCR therapies have significant shortcomings. To this end, we are developing BPX-401 for hematological cancers expressing the CD19 antigen, BPX-601 for solid tumors overexpressing PSCA and BPX-701 for solid tumors expressing PRAME. We believe that these product candidates may address serious safety concerns associated with conventional CAR-T and TCR therapies and achieve higher overall potency and efficacy, thereby widening the therapeutic window compared to other CAR-T and TCR product candidates. We intend to dedicate significant resources in the near term to advance BPX-401, BPX-601 and BPX-701 as well as our other product candidates toward human proof-of-concept data.

- n ***Selectively pursue partnerships and collaborations.*** Although our priority is to develop internal product candidates, we may pursue opportunistic partnerships and collaborations for our technologies, including CaspaCIDE and DeCIDE. In indications outside of our interest or expertise, we may structure transactions in which our molecular switches are incorporated into our partners' CAR-T or TCR product candidates. We intend to build on our existing strong relationships with premier cancer research centers around the world to identify new opportunities and position our company at the forefront of innovations in the field of cellular immunotherapy.

- n ***Continue to innovate around our proprietary CID platform.*** We believe that our CID platform can be further leveraged to discover other novel technologies and therapeutic applications to capitalize on additional market opportunities. We intend to evaluate BPX-201 and other product candidates based on our DeCIDE technology in combination with other cancer immunotherapy such as checkpoint inhibitors. We are also developing new switches and two-switch systems to provide greater control over cellular immunotherapy.

- n ***Continue to strengthen our intellectual property profile.*** We believe that having a comprehensive patent estate that provides strong barriers to entry is critical to the success of our business. As such, our management team has made a concerted effort to develop and secure our intellectual property since inception. We currently own or have exclusive licenses to 74 issued patents and 45 pending patent applications. These patents and patent applications include composition and/or method of use claims in the United States, Europe and other jurisdictions. We intend to continue to strengthen our patent estate by developing and filing for patents on various aspects of our technologies and product candidates as well as through in-licensing activities with research institutions and other biopharmaceutical companies.

- n ***Become a fully integrated cellular immunotherapy company.*** Developing product candidates for cellular immunotherapy is complex and requires significant in-house capabilities in various areas of drug

development. Over the years we have built a solid foundation from which to fulfill the highly demanding clinical and regulatory requirements of genetically modified cellular immunotherapy, with expertise in research and discovery, clinical trial management, data analysis, manufacturing, quality assurance and regulatory affairs. We intend to use a portion of the net proceeds from this offering to continue hiring staff with necessary expertise and investing in infrastructure to support the growth of our clinical development activities and to enable us to become the leading cellular immunotherapy company.

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Recent Developments

To enable further development of our proprietary technology and product candidates, we completed a private placement of \$55 million of Series C convertible preferred stock in August 2014. Investors in the transaction included, among others, Baker Brothers, RA Capital Management, LLC, Perceptive Advisors, LLC, Jennison Associates LLC (on behalf of certain clients), Sabby Capital, LLC, Ridgeback Capital Management, venBio Select, Redmile Group, LLC and AJU IB Investment, as well as our then current investors, including AVG Ventures and Remeditex Ventures.

Certain aspects of our platform technology are licensed from ARIAD Pharmaceuticals, Inc., or ARIAD. In October 2014, we amended our license agreement with ARIAD, pursuant to which we agreed to pay ARIAD \$50 million in three tranches payments, including an initial payment of \$15 million in connection with the execution of the amendment. In exchange, ARIAD gave us a fully paid-up license to its cell-signaling technology and agreed to return of all of the 677,463 shares of our common stock currently held by ARIAD at the time of the second tranche payment. The scope of the license and the field of use were also expanded as part of the amendment. The amended agreement gives us a worldwide exclusive license to ARIAD's cell-signaling technology for broad use in human cell therapies for all diseases on a royalty- and milestone-free basis. See [Business Our License Agreements](#).

Risks Associated With Our Business

Our business is subject to numerous risks, as more fully described in the section entitled [Risk Factors](#) immediately following this prospectus summary. You should read these risks before you invest in our common stock. We may be unable, for many reasons, including those that are beyond our control, to implement our business strategy. In particular, risks associated with our business include:

- n We have incurred net losses in every year since our inception and anticipate that we will continue to incur net losses in the future.
- n Biopharmaceutical product development is a highly speculative undertaking and involves a substantial degree of uncertainty. We have never generated any revenue from product sales and may never be profitable.
- n We have concentrated our therapeutic product research and development efforts on our CID platform, a novel therapeutic approach, and our future success depends on the successful development of this therapeutic approach.
- n Our clinical trials may fail to demonstrate adequately the safety and efficacy of any of our product candidates, which would prevent or delay regulatory approval and commercialization.
- n We may not be successful in our efforts to use and expand our CID platform to build a pipeline of product candidates and develop marketable products.

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The FDA regulatory approval process is lengthy and time-consuming, and we may experience significant delays in the clinical development and regulatory approval of our product candidates. Further, the FDA may disagree with our regulatory plans and we may fail to obtain regulatory approval of our product candidates.

- n Due to the novel nature of our technology and the potential for our product candidates to offer therapeutic benefit in a single administration, we face uncertainty related to pricing and reimbursement for these product candidates. Coverage and reimbursement may be limited or unavailable in certain market segments for our product candidates, which could make it difficult for us to sell our product candidates profitably.

- n We depend on intellectual property licensed from third parties and termination of any of these licenses could result in the loss of significant rights, which would harm our business.

- n We have identified a material weakness in our internal control over financial reporting. If our internal control over financial reporting is not effective, we may not be able to accurately report our financial results or file our periodic reports in a timely manner, which may cause investors to lose confidence in our reported financial information and may lead to a decline in our stock price.

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Corporate Information

We were incorporated in Delaware in July 2004. Our principal executive offices are located at 2130 W. Holcombe Blvd., Ste. 800, Houston, Texas and our telephone number is (832) 384-1100. Our corporate website address is www.bellicum.com. Information contained on or accessible through our website is not a part of this prospectus, and the inclusion of our website address in this prospectus is an inactive textual reference only.

Implications of Being an Emerging Growth Company

As a company with less than \$1.0 billion in revenue during our last fiscal year, we qualify as an emerging growth company as defined in the Jumpstart Our Business Startups Act, or the JOBS Act, enacted in April 2012. An emerging growth company may take advantage of reduced reporting requirements that are otherwise applicable to public companies. These provisions include, but are not limited to:

- n being permitted to present only two years of audited financial statements and only two years of related Management's Discussion and Analysis of Financial Condition and Results of Operations in this prospectus;
- n not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, as amended;
- n reduced disclosure obligations regarding executive compensation in our periodic reports, proxy statements and registration statements; and
- n exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved.

We may use these provisions until the last day of our fiscal year following the fifth anniversary of the closing of this offering. However, if certain events occur prior to the end of such five-year period, including if we become a large accelerated filer, our annual gross revenue exceed \$1.0 billion or we issue more than \$1.0 billion of non-convertible debt in any three-year period, we will cease to be an emerging growth company prior to the end of such five-year period.

We have elected to take advantage of certain of the reduced disclosure obligations in the registration statement of which this prospectus is a part and may elect to take advantage of other reduced reporting requirements in future filings. As a result, the information that we provide to our stockholders may be different than you might receive from other public reporting companies in which you hold equity interests.

The JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. We have irrevocably elected not to avail ourselves of this exemption and, therefore, we will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

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THE OFFERING

Common stock offered by us	7,350,000 shares
Common stock to be outstanding after this offering	25,849,571 shares
Option to purchase additional shares	We have granted to the underwriters the option, exercisable for 30 days from the date of this prospectus, to purchase up to 1,102,500 additional shares of common stock.
Use of proceeds	We estimate that we will receive net proceeds of approximately \$127.1 million (or approximately \$146.5 million if the underwriters option to purchase additional shares is exercised in full) from the sale of the shares of common stock offered by us in this offering, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us. We intend to use the net proceeds from this offering for the following purposes: (1) \$21.0 million to fund our ongoing and planned Phase 1/2 clinical trials of BPX-501, (2) \$30.0 million to fund pre-clinical and Phase 1/2 clinical trial of BPX-401, BPX-601 and BPX-701 as well as preclinical development of our other CART and TCR programs, (3) \$4.0 million to fund our ongoing Phase 1/2 clinical trial and our planned Phase 1/2 clinical trial of BPX-201 in combination with checkpoint inhibitors, (4) \$11.0 million to fund the construction of tenant improvements and the purchase of capital equipment at our Houston facility, and (5) the remainder to fund other working capital purposes, including general operating expenses. See Use of Proceeds.
Risk factors	You should read the Risk Factors section of this prospectus for a discussion of certain of the factors to consider carefully before deciding to purchase any shares of our common stock.
NASDAQ Global Market symbol	BLCM
The number of shares of our common stock to be outstanding after this offering is based on 2,124,386 shares of common stock outstanding as of September 30, 2014, and assumes:	

n the issuance by us of 7,350,000 shares of our common stock in this offering;

- n the conversion of all of our convertible preferred stock outstanding into an aggregate of 12,224,819 shares of common stock upon the closing of this offering;
- n the net exercise of outstanding warrants to purchase common stock for an aggregate of 114,468 shares of common stock;
- n that all of the holders of Series B convertible preferred stock will elect to have their accrued dividends converted into common stock at the time of conversion of their shares of Series B convertible preferred stock into shares of common stock in connection with this offering, which will result in the issuance by us of 177,349 shares of common stock;
- n the issuance by us of 6,559,598 shares of Series C convertible preferred stock issuable upon the exercise of warrants issued by us in August 2014, pursuant to that certain Series C Preferred Stock and Warrant Purchase Agreement, or the Series C Purchase Agreement and the conversion of these shares of Series C convertible preferred stock into an aggregate of 3,858,549 shares of common stock;

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and excludes:

- n 1,602,339 shares of common stock issuable upon the exercise of outstanding stock options as of September 30, 2014, at a weighted-average exercise price of \$2.33 per share;
- n 2,956,909 shares of our common stock reserved for future issuance under our 2014 equity incentive plan, or the 2014 Plan, which will become effective as of the date of the effectiveness of the registration statement of which this prospectus forms a part, which number includes the 258,823 shares subject to stock options and a stock award that will be granted upon the effective date of the 2014 Plan and includes the 1,382,481 shares of common stock reserved for issuance under our 2011 stock option plan, as amended, or the 2011 Plan as of September 30, 2014, reduced by the 1,031,454 shares of common stock issuable upon the exercise of the stock options granted under the 2011 Plan subsequent to September 30, 2014, which aggregate of 351,027 shares will be added to the shares reserved under the 2014 Plan when the 2014 Plan becomes effective;
- n 550,000 shares of common stock reserved for future issuance under our 2014 Employee Stock Purchase Plan, or the ESPP, which will become effective upon the execution and delivery of the underwriting agreement for this offering; and
- n 355,392 shares of common stock issuable upon the exercise of outstanding warrants as of September 30, 2014, at an exercise price of \$0.0017 per share.

Unless otherwise indicated, all information contained in this prospectus assumes or gives effect to:

- n a 1-for-1.7 reverse stock split of our common stock effected on December 5, 2014;
- n the filing of our amended and restated certificate of incorporation and the adoption of our amended and restated bylaws immediately prior to the closing of this offering; and
- n no exercise by the underwriters of their option to purchase up to an additional 1,102,500 shares of our common stock.

Certain of our existing stockholders and their affiliated entities have indicated an interest in purchasing up to approximately \$50.0 million of shares of our common stock in this offering at the public offering price. However, because indications of interest are not binding agreements or commitments to purchase, these entities may determine to purchase more or fewer shares than they have indicated or not to purchase any shares in this offering.

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The following summary financial data should be read together with our financial statements and related notes, Selected Financial Data and Management's Discussion and Analysis of Financial Condition and Results of Operations appearing elsewhere in this prospectus. The summary financial data in this section are not intended to replace our financial statements and the related notes. We derived the summary statement of operations data for the years ended December 31, 2012 and 2013 from our audited financial statements and related notes appearing elsewhere in this prospectus. We derived the summary statement of operations data for the nine months ended September 30, 2013 and 2014 and the summary balance sheet data as of September 30, 2014, from our unaudited financial statements and related notes appearing elsewhere in this prospectus. The unaudited financial data, in management's opinion, have been prepared on the same basis as the audited financial statements and related notes included elsewhere in this prospectus, and include all adjustments, consisting only of normal recurring adjustments, that management considers necessary for a fair presentation of the information for the periods presented. Our historical results are not necessarily indicative of the results that may be expected in the future, and results from our interim period may not necessarily be indicative of the results of the entire year.

(in thousands, except share and per share data)	YEAR ENDED DECEMBER 31,		NINE MONTHS ENDED SEPTEMBER 30,	
	2012	2013	2013 (unaudited)	2014 (unaudited)
Statement of Operations Data:				
Grant revenue	\$ 1,470	\$ 1,941	\$ 1,122	\$ 1,766
Operating expenses:				
Research and development	5,796	7,050	4,564	7,078
General and administrative	1,943	2,813	1,997	3,135
Total operating expenses	7,739	9,863	6,561	10,213
Loss from operations	(6,269)	(7,922)	(5,439)	(8,447)
Other income (expense):				
Interest income	7	4	2	15
Interest expense	(1)	(51)	(38)	(38)
Change in value of warrant liability				(1,197)
Total other income (expense)	6	(47)	(36)	(1,220)
Net loss	\$ (6,263)	\$ (7,969)	\$ (5,475)	\$ (9,667)
Preferred stock dividends	(757)	(1,094)	(695)	(1,432)

Net loss available to common stockholders	\$ (7,020)	\$ (9,063)	\$ (6,170)	\$ (11,099)
Net loss per share, basic and diluted ⁽¹⁾	\$ (4.26)	\$ (5.25)	\$ (3.58)	\$ (5.45)
Weighted-average shares outstanding, basic and diluted	1,648,198	1,725,992	1,725,992	2,036,025
Pro forma net loss (unaudited)		\$ (7,969)		\$ (9,667)
Pro forma net loss per share, basic and diluted (unaudited) ⁽²⁾		\$ (1.32)		\$ (0.98)
Pro forma weighted-average shares outstanding, basic and diluted (unaudited) ⁽²⁾		6,051,619		9,827,767

⁽¹⁾ See Note 2 to our financial statements appearing elsewhere in this prospectus for an explanation of the method used to calculate the basic and diluted net loss per common share and the number of shares used in the computation of the per share amounts.

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- (2) The calculations for the unaudited pro forma net loss per common share, basic and diluted, assume (1) the conversion of all our outstanding shares of convertible preferred stock as of September 30, 2014, into an aggregate of 12,224,819 shares of our common stock, (2) the net exercise of outstanding warrants to purchase common stock (which will expire upon the closing of this offering if not exercised) into 114,468 shares of our common stock, (3) the issuance of 6,559,598 shares of Series C convertible preferred stock upon the exercise of warrants, and the conversion of such shares into 3,858,549 shares of common stock in connection with the closing of this offering. The calculations exclude the impact of the issuance by us of an aggregate of 177,349 shares of our common stock as payment of the accrued dividend payable to the holders of Series B convertible preferred stock in connection with this offering.

(in thousands)	AS OF SEPTEMBER 30, 2014		
	ACTUAL (unaudited)	PRO FORMA ⁽¹⁾⁽²⁾	PRO FORMA AS ADJUSTED ⁽³⁾
Balance Sheet Data:			
Cash and cash equivalents	\$ 61,932	\$ 86,287	\$ 213,469
Working capital	49,849	65,269	192,369
Total assets	66,331	90,686	217,786
Convertible preferred stock	90,753		
Accumulated deficit	(38,646)	(83,559)	(83,559)
Total stockholders' (deficit) equity	(38,794)	51,913	179,013

- (1) Pro forma amounts reflect (1) the conversion of all our outstanding shares of our convertible preferred stock as of September 30, 2014 into an aggregate of 12,224,819 shares of our common stock in connection with the closing of this offering, and (2) the net exercise of outstanding warrants to purchase shares of our common stock (which will expire upon the closing of this offering if not exercised) into 114,468 shares of our common stock, (3) the issuance by us of 177,349 shares of common stock, as payment of the accrued dividend on the outstanding shares of Series B convertible preferred stock payable to the holders of Series B convertible preferred stock in connection with this offering, (4) the issuance of 6,559,598 shares of Series C convertible preferred stock upon the exercise of warrants, and the conversion of such shares into 3,858,549 shares of common stock in connection with the closing of this offering.
- (2) Pro forma amounts reflect the October 2014 amendment to our license agreement with Ariad, pursuant to which we agreed to pay \$50.0 million in exchange for expanded use of the license and the termination of all obligations to make milestone and royalty payments to Ariad in the future. We have reflected (1) a decrease in cash for the initial payment of \$15.0 million in October 2014, (2) a liability of \$35.0 million to recognize the promissory note for the remaining balance, (3) a \$5.1 million reduction in equity to reflect the estimated fair value, as of October 2014, of the common stock to be returned to us in connection with the second payment to Ariad and (4) a \$44.9 million charge to research and development expense. The final accounting treatment is still under review and may

ultimately result in different accounting treatment, including discounting the liability or less research and development expense.

- (3) Pro forma as adjusted amounts reflect the pro forma conversion adjustments described in footnotes (1) and (2) above, as well as the sale of 7,350,000 shares of our common stock in this offering at the initial public offering price of \$19.00 per share after deducting the underwriting discounts and commissions and estimated offering expenses payable by us.

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RISK FACTORS

Investing in our common stock involves a high degree of risk. You should carefully consider the risks described below, as well as the other information in this prospectus, including our financial statements and the related notes and Management's Discussion and Analysis of Financial Condition and Results of Operations, before deciding whether to invest in our common stock. The occurrence of any of the events or developments described below could harm our business, financial condition, results of operations and growth prospects. In such an event, the market price of our common stock could decline and you may lose all or part of your investment. Additional risks and uncertainties not presently known to us or that we currently deem immaterial also may impair our business operations.

Risks Related to Our Business and Industry

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

We are a clinical stage biopharmaceutical company with a limited operating history. We are not profitable and have incurred losses in each period since our inception in 2004. To date, we have financed our operations primarily through private placements of convertible debt and preferred stock. For the years ended December 31, 2012 and 2013, we reported a net loss of \$6.3 million and \$8.0 million, respectively. For the nine months ended September 30, 2013 and 2014, we reported a net loss of \$5.5 million and \$9.7 million, respectively. As of September 30, 2014, we had an accumulated deficit of \$38.6 million. We expect to continue to incur significant losses for the foreseeable future, and we expect these losses to increase as we continue our research and development of, and seek regulatory approvals for, our product candidates.

Even if we succeed in commercializing one or more of our product candidates, we will continue to incur substantial research and development and other expenditures to develop and market additional product candidates. We may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business. The size of our future net losses will depend, in part, on the rate of future growth of our expenses and our ability to generate revenue. Our prior losses and expected future losses have had and will continue to have an adverse effect on our stockholders' equity and working capital.

Biopharmaceutical product development is a highly speculative undertaking and involves a substantial degree of uncertainty. We have never generated any revenue from product sales and may never be profitable.

We have devoted substantially all of our financial resources and efforts to developing our proprietary CID technology platform, identifying potential product candidates and conducting preclinical studies and clinical trials. We are still in the early stages of developing our product candidates, and we have not completed development of any products. Our ability to generate revenue and achieve profitability depends in large part on our ability, alone or with partners, to successfully complete the development of, obtain the necessary regulatory approvals for, and commercialize, product candidates. We do not anticipate generating revenues from sales of products for the foreseeable future. Our ability to generate future revenues from product sales depends heavily on our success in:

- n completing clinical trials through all phases of clinical development of our current product candidates, as well as the product candidates that are being developed by our partners and licensees;

- n seeking and obtaining marketing approvals for product candidates that successfully complete clinical trials;
- n launching and commercializing product candidates for which we obtain marketing approval, with a partner or, if launched independently, successfully establishing a sales force, marketing and distribution infrastructure;
- n identifying and developing new product candidates;
- n progressing our pre-clinical programs into human clinical trials;
- n establishing and maintaining supply and manufacturing relationships with third parties;
- n developing new molecular switches based on our proprietary CID technology platform;
- n maintaining, protecting, expanding and enforcing our intellectual property; and
- n attracting, hiring and retaining qualified personnel.

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Because of the numerous risks and uncertainties associated with biologic product development, we are unable to predict the timing or amount of increased expenses and when we will be able to achieve or maintain profitability, if ever. In addition, our expenses could increase beyond expectations if we are required by the FDA, or foreign regulatory agencies, to perform studies and clinical trials in addition to those that we currently anticipate, or if there are any delays in our or our partners completing clinical trials or the development of any of our product candidates. If one or more of the product candidates that we independently develop is approved for commercial sale, we anticipate incurring significant costs associated with commercializing such product candidates. Even if we or our partners are able to generate revenues from the sale of any approved products, we may not become profitable and may need to obtain additional funding to continue operations, which may not be available to us on favorable terms, if at all. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would depress the value of our company and could impair our ability to raise capital, expand our business, maintain our research and development efforts, diversify our product offerings or even continue our operations. A decline in the value of our company could also cause you to lose all or part of your investment.

We have concentrated our therapeutic product research and development efforts on our CID platform, and our future success depends on the successful development of this therapeutic approach.

Our proprietary CID technology platform is novel and there are no approved products or product candidates in late-stage clinical trials based on this technology. Additionally, the safety and efficacy profile of rimiducid has not been subject to large scale clinical testing. If rimiducid is found to have a poor safety profile in clinical trials, or if our technology is not effective, we may be required to redesign all of our product candidates, which would require significant time and expense. In addition, our CID platform technology may not be applicable or effective in the development of additional cellular immunotherapies beyond our current programs which would adversely affect our business and prospects.

CAR T cell therapies are novel and present significant challenges.

CAR-T and TCR product candidates represent a relatively new field of cellular immunotherapy and there are no FDA-approved products in this area. Advancing this novel and personalized therapy creates significant challenges for us, including:

- n obtaining regulatory approval, as the FDA and other regulatory authorities have limited experience with commercial development of T-cell therapies for cancer;
- n sourcing clinical and, if approved, commercial supplies for the materials used to manufacture and process our product candidates;
- n developing a consistent and reliable process, while limiting contamination risks, for engineering and manufacturing T cells *ex vivo* and infusing the engineered T cells into the patient;
- n educating medical personnel regarding the potential safety benefits, as well as the challenges, of incorporating our product candidates into their treatment regimens; and

n establishing sales and marketing capabilities upon obtaining any regulatory approval to gain market acceptance of a novel therapy.

Our inability to successfully develop CAR-T and TCR cell therapies or develop processes related to the manufacture, sales and marketing of these therapies would adversely affect our business, results of operations and prospects. We believe that we have appropriately accounted for the above factors while pursuing the development and commercialization of our product candidates, but we cannot entirely eliminate the risks associated with novel technology.

Failure to successfully develop and obtain approval of our lead product candidate BPX-501 or our other clinical product candidates could adversely affect our future success.

Our business and future success depends, in part, on our ability to obtain regulatory approval of and then successfully commercialize our lead product candidate, BPX-501 and our other clinical product candidates. BPX-501 is in the early stages of development. All of our product candidates, including BPX-501, will require additional clinical and non-clinical development, regulatory review and approval in multiple jurisdictions, substantial investment, access to sufficient commercial manufacturing capacity and significant marketing efforts before we can generate any revenue from product sales. In addition, because BPX-501 is our most advanced product candidate,

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and because many of our other product candidates are based on similar technology, if BPX-501 encounters safety or efficacy problems, developmental delays or regulatory issues or other problems, our development plans and business for our other product candidates would be significantly harmed. In addition, our product candidates that incorporate the CID safety switch combine genetically modified T cells that are used to enhance the patients immune system and a small molecule that leads to the death of these modified T cells if they cause safety issues.

Our clinical trials may fail to adequately demonstrate the safety and efficacy of any of our product candidates, which would prevent or delay regulatory approval and commercialization.

Before obtaining regulatory approvals for the commercial sale of our product candidates, we must demonstrate through lengthy, complex and expensive preclinical testing and clinical trials that our product candidates are both safe and effective for use in each target indication. Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical trial process. The results of preclinical studies and early clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials. We expect there may be greater variability in results for cellular immunotherapy products processed and administered on a patient-by-patient basis, like all of our CID technology-based development and product candidates, than for off-the-shelf products, like many drugs. There is typically an extremely high rate of attrition from the failure of product candidates proceeding through clinical trials. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy profile despite having progressed through preclinical studies and initial clinical trials. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or unacceptable safety issues, notwithstanding promising results in earlier clinical trials. Most product candidates that commence clinical trials are never approved as products.

We have not completed any clinical studies of our current product candidates. Success in early clinical studies may not be indicative of results obtained in later studies.

Many of our current product candidates have not initiated evaluation in human clinical studies, and we may experience unexpected results in the future. Differences in cell processing, time of administration and patient conditioning, among other factors, may result in our experiencing different results in our clinical trials from those reported in trials by our collaborators, and may mean that we experience different results in our clinical trials. In addition, data from preclinical studies and investigator-led Phase 1 or Phase 1/2 clinical trials of BPX-501 therapy should not be relied upon as evidence that later or later-scale clinical trials will succeed. We have designed our planned Phase 1/2 single-arm multicenter clinical trial of BPX-501 primarily to assess safety and efficacy in a small number of adult patients with malignant disease. In addition, we are initiating additional Phase 1 and Phase 1/2 clinical trials of BPX-501 and there are a number of investigator-led clinical trials of BPX-501 ongoing and planned.

Similarly, results from preclinical studies, such as *in vitro* and *in vivo* studies, of BPX-401, BPX-601, BPX-701 and our other preclinical programs may not be indicative of the results of clinical trials of these product candidates. Furthermore, we may not be able to commence human clinical trials on any of our preclinical product candidates on the time frames we expect. Our failure to meet these expected targets would likely have an adverse effect on our stock price.

Even if the clinical trials are successfully completed, we cannot guarantee that the FDA or foreign regulatory authorities will interpret the results as we do, and more clinical trials could be required before we submit our product candidates for approval. To the extent that the results of the clinical trials are not satisfactory to the FDA or foreign regulatory authorities for support of a marketing application, approval of our product candidates may be significantly delayed, or we may be required to expend significant additional resources, which may not be available to us, to conduct additional clinical trials in support of potential approval of our product candidates.

We may not be successful in our efforts to use and expand our CID platform to build a pipeline of product candidates and develop marketable products.

We believe that our CID platform, which serves as the foundation of our CaspaCIDE, CIDE CAR, GoCAR-T and DeCIDE technologies, can be further leveraged to discover other novel technologies, therapeutic applications and market opportunities. For example, we are currently conducting research in applying our platform TCR therapies for solid tumors, where immune toxicities associated with treatment are even more severe than CAR-T therapies. We are also developing new molecular switches and two-switch systems to provide greater control over cellular immunotherapy.

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We are at a very early stage of development and our platform has not yet, and may never lead to, approved or marketable products. Even if we are successful in continuing to build our pipeline, the potential product candidates that we identify may not be suitable for clinical development, including for reasons related to their harmful side effects, limited efficacy or other characteristics that indicate that they are unlikely to be products that will receive marketing approval and achieve market acceptance. If we do not successfully develop and commercialize product candidates based upon our technological approach, we may not be able to obtain product or partnership revenues in future periods, which would adversely affect our business, prospects, financial condition and results of operations.

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

We depend and will continue to depend upon independent investigators and collaborators, such as universities, medical institutions, and strategic partners to conduct our preclinical and clinical trials under agreements with us. Negotiations of budgets and contracts with study sites may result in delays to our development timelines and increased costs. We will rely heavily on these third parties over the course of our clinical trials, and we control only certain aspects of their activities. Nevertheless, we are responsible for ensuring that each of our studies is conducted in accordance with applicable protocol, legal, regulatory and scientific standards, and our reliance on third parties does not relieve us of our regulatory responsibilities. We and these third parties are required to comply with current good clinical practices, or cGCPs, which are regulations and guidelines enforced by the FDA and comparable foreign regulatory authorities for product candidates in clinical development. Regulatory authorities enforce these cGCPs through periodic inspections of clinical trial sponsors, principal investigators and clinical trial sites. If we or any of these third parties fail to comply with applicable cGCP regulations, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot assure you that, upon inspection, such regulatory authorities will determine that any of our clinical trials comply with the cGCP regulations. In addition, our clinical trials must be conducted with biologic product produced under current good manufacturing practices, or cGMPs, regulations and will require a large number of test patients. Our failure or any failure by these third parties to comply with these regulations or to recruit a sufficient number of patients may require us to repeat clinical trials, which would delay the regulatory approval process. Moreover, our business may be implicated if any of these third parties violates federal or state fraud and abuse or false claims laws and regulations or healthcare privacy and security laws.

Any third parties conducting our clinical trials are and will not be our employees and, except for remedies available to us under our agreements with these third parties, we cannot control whether or not they devote sufficient time and resources to our ongoing preclinical, clinical and nonclinical programs. These third parties may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other drug development activities, which could affect their performance on our behalf. If these third parties do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements or for other reasons, our clinical trials may be extended, delayed or terminated and we may not be able to complete development of, obtain regulatory approval of or successfully commercialize our product candidates. As a result, our financial results and the commercial prospects for our product candidates would be harmed, our costs could increase and our ability to generate revenue could be delayed.

Switching or adding third parties to conduct our clinical trials involves substantial cost and requires extensive management time and focus. In addition, there is a natural transition period when a new third party commences work. As a result, delays occur, which can materially impact our ability to meet our desired clinical development timelines.

Additionally, we are conducting multiple clinical trials in Europe and may plan additional testing of our technology and product candidates in other foreign jurisdictions. We currently have limited staffing and capabilities in foreign countries, and may not be able to effectively resolve potential disputes with our independent investigators and collaborators.

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If we encounter difficulties enrolling patients in our clinical trials, our clinical development activities could be delayed or otherwise adversely affected.

We may experience difficulties in patient enrollment in our clinical trials for a variety of reasons. The timely completion of clinical trials in accordance with their protocols depends, among other things, on our ability to enroll a sufficient number of patients who remain in the study until its conclusion. The enrollment of patients depends on many factors, including:

- n the patient eligibility criteria defined in the protocol;
- n the size of the patient population required for analysis of the trial's primary endpoints;
- n the proximity of patients to study sites;
- n the design of the clinical trial;
- n our ability to recruit clinical trial investigators with the appropriate competencies and experience;
- n our ability to obtain and maintain patient consents; and

n the risk that patients enrolled in clinical trials will drop out of the clinical trials before completion. In particular, some of our clinical trials will look to enroll patients with characteristics which are found in a very small population, such as patients with CD19-expressing cancers, such as ALL, CLL and non-Hodgkin's lymphomas, and patients with orphan inherited blood disorders. Our clinical trials will compete with other clinical trials for product candidates that are in the same therapeutic areas as our product candidates, and this competition will reduce the number and types of patients available to us, because some patients who might have opted to enroll in our clinical trials may instead opt to enroll in a trial being conducted by one of our competitors. Since the number of qualified clinical investigators is limited, we expect to conduct some of our clinical trials at the same clinical trial sites that some of our competitors use, which will reduce the number of patients who are available for our clinical trials in these clinical trial sites. Moreover, because our product candidates represent a departure from more commonly used methods for cancer treatment, potential patients and their doctors may be inclined to use conventional therapies, such as chemotherapy and antibody therapy, rather than enroll patients in any of our future clinical trials. Patients may also be unwilling to participate in our clinical trials because of negative publicity from adverse events in the biotechnology or gene therapy industries.

Delays in patient enrollment may result in increased costs or may affect the timing or outcome of the planned clinical trials, which could prevent completion of these clinical trials and adversely affect our ability to advance the development of our product candidates.

Any adverse developments that occur during any clinical trials conducted by academic investigators, our collaborators or other entities conducting clinical trials under independent INDs may affect our ability to obtain regulatory approval or commercialize our product candidates.

BPX-501 and certain of our other CaspaCIDE product candidates are being used by third parties in clinical trials for which we are collaborating or in clinical trials which are completely independent of our development program. We have little to no control over the conduct of such clinical trials. If serious adverse events occur during these or any other clinical trials using our product candidates, the FDA and other regulatory authorities may delay, limit or deny approval of our product candidate or require us to conduct additional clinical trials as a condition to marketing approval, which would increase our costs. If we receive FDA approval for BPX-501 or any other CaspaCIDE product candidate and a new and serious safety issue is identified in connection with clinical trials conducted by third parties, the FDA and other regulatory authorities may withdraw their approval of the product or otherwise restrict our ability to market and sell our product. In addition, treating physicians may be less willing to administer our product due to concerns over such adverse events, which would limit our ability to commercialize our product.

Adverse side effects or other safety risks associated with our product candidates could delay or preclude approval, cause us to suspend or discontinue clinical trials, abandon product candidates, limit the commercial profile of an approved label, or result in significant negative consequences following marketing approval, if any.

Undesirable side effects caused by our product candidates could result in the delay, suspension or termination of clinical trials by us, the FDA or other regulatory authorities for a number of reasons. If we elect or are required to delay, suspend or terminate any clinical trial of any product candidates that we develop, the commercial prospects of such product candidates will be harmed and our ability to generate product revenues from any of these product

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candidates will be delayed or eliminated. Serious adverse events observed in clinical trials could hinder or prevent market acceptance of the product candidate at issue. Any of these occurrences may harm our business, prospects, financial condition and results of operations significantly.

In other clinical trials involving CAR T cells, the most prominent acute toxicities included symptoms thought to be associated with the release of cytokines, such as fever, low blood pressure and kidney dysfunction. Some patients also experienced toxicity of the central nervous system, such as confusion, cranial nerve dysfunction and speech impairment. Adverse events by worst grade and attributed to CAR T cells were severe and life threatening in some patients. The life threatening events were related to kidney dysfunction and toxicities of the central nervous system. Severe and life threatening toxicities occurred mostly in the first two weeks after cell infusion and generally resolved within three weeks. In the past, several patients have also died in clinical trials by others involving CAR T cells.

Clinical trials are expensive, time-consuming and difficult to design and implement.

Human clinical trials are expensive and difficult to design and implement, in part because they are subject to rigorous regulatory requirements. Because our product candidates are based on new technology and engineered on a patient-by-patient basis, we expect that they will require extensive research and development and have substantial manufacturing and processing costs. In addition, costs to treat patients with relapsed/refractory cancer and to treat potential side effects that may result from therapies such as our current and future product candidates can be significant. Accordingly, our clinical trial costs are likely to be significantly higher than for more conventional therapeutic technologies or drug products. In addition, our proposed personalized product candidates involve several complex and costly manufacturing and processing steps, the costs of which will be borne by us. The costs of our clinical trials may increase if the FDA does not agree with our clinical development plans or requires us to conduct additional clinical trials to demonstrate the safety and efficacy of our product candidates.

We face significant competition from other biotechnology and pharmaceutical companies, and our operating results will suffer if we fail to compete effectively.

The biopharmaceutical industry is characterized by intense competition and rapid innovation. Our competitors may be able to develop other compounds or drugs that are able to achieve similar or better results. Our potential competitors include major multinational pharmaceutical companies, established biotechnology companies, specialty pharmaceutical companies and universities and other research institutions. Many of our competitors have substantially greater financial, technical and other resources, such as larger research and development staff and experienced marketing and manufacturing organizations and well-established sales forces. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large, established companies. Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated in our competitors. Competition may increase further as a result of advances in the commercial applicability of technologies and greater availability of capital for investment in these industries. Our competitors, either alone or with collaborative partners, may succeed in developing, acquiring or licensing on an exclusive basis drug or biologic products that are more effective, safer, more easily commercialized or less costly than our product candidates or may develop proprietary technologies or secure patent protection that we may need for the development of our technologies and products. We believe the key competitive factors that will affect the development and commercial success of our product candidates are efficacy, safety, tolerability, reliability, convenience of use, price and reimbursement.

Specifically, genetically engineering T cells faces significant competition in both the CAR and TCR technology space from multiple companies, including Adaptimmune Limited, bluebird bio, Inc., Celgene Corporation, Collectis SA, GlaxoSmithKline plc, Intrexon Corporation, Juno Therapeutics, Inc., Kite Pharma, Inc., Novartis AG and Pfizer Inc.

Even if we obtain regulatory approval of our product candidates, the availability and price of our competitors' products could limit the demand and the price we are able to charge for our product candidates. We may not be able to implement our business plan if the acceptance of our product candidates is inhibited by price competition or the reluctance of physicians to switch from existing methods of treatment to our product candidates, or if physicians switch to other new drug or biologic products or choose to reserve our product candidates for use in limited circumstances. For additional information regarding our competition, see Business Competition.

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We are highly dependent on our key personnel, and if we are not successful in attracting and retaining highly qualified personnel, we may not be able to successfully implement our business strategy.

Our ability to compete in the highly competitive biotechnology and pharmaceutical industries depends upon our ability to attract and retain highly qualified managerial, scientific and medical personnel. We are highly dependent on our management, scientific and medical personnel, including our Chief Executive Officer, our Chief Operating Officer, our Chief Medical Officer and Chief Technology Officer, and our Chief Scientific Officer. The loss of the services of any of our executive officers, other key employees, and other scientific and medical advisors, and our inability to find suitable replacements could result in delays in product development and harm our business.

To induce valuable employees to remain at our company, in addition to salary and cash incentives, we have provided stock options that vest over time. The value to employees of stock options that vest over time may be significantly affected by movements in our stock price that are beyond our control, and may at any time be insufficient to counteract more lucrative offers from other companies. Despite our efforts to retain valuable employees, members of our management, scientific and development teams may terminate their employment with us on short notice. Although we have employment agreements with our key employees, these employment agreements provide for at-will employment, which means that any of our employees could leave our employment at any time, with or without notice. We do not maintain key man insurance policies on the lives of these individuals or the lives of any of our other employees. Our success also depends on our ability to continue to attract, retain and motivate highly skilled scientific and medical personnel.

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

As of September 30, 2014, we had 30 employees, all of whom were full-time. As our development and commercialization plans and strategies develop, and as we transition into operating as a public company, we expect to need additional managerial, operational, sales, marketing, financial and other personnel. Future growth would impose significant added responsibilities on members of management, including:

- n identifying, recruiting, integrating, maintaining and motivating additional employees, including a Chief Financial Officer;
- n managing our internal development efforts effectively, including the clinical and FDA review process for our product candidates, while complying with our contractual obligations to contractors and other third parties; and
- n improving our operational, financial and management controls, reporting systems and procedures.

There are a small number of individuals with experience in cell therapy and the competition for such individuals is high. Our future financial performance and our ability to commercialize our product candidates will depend, in part, on our ability to effectively manage any future growth, and our management may also have to divert a disproportionate amount of its attention away from day-to-day activities in order to devote a substantial amount of time to managing these growth activities. We currently rely, and for the foreseeable future will continue to rely, in substantial part on certain independent organizations, advisors and consultants to provide certain services, including substantially all aspects of regulatory approval, clinical management, and manufacturing. There can be no assurance that the services of independent organizations, advisors and consultants will continue to be available to us on a timely

basis when needed, or that we can find qualified replacements. In addition, if we are unable to effectively manage our outsourced activities or if the quality or accuracy of the services provided by consultants is compromised for any reason, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval of our product candidates or otherwise advance our business. There can be no assurance that we will be able to manage our existing consultants or find other competent outside contractors and consultants on economically reasonable terms, or at all.

If we are not able to effectively expand our organization by hiring new employees and expanding our groups of consultants and contractors, we may not be able to successfully implement the tasks necessary to further develop and commercialize our product candidates and, accordingly, may not achieve our research, development and commercialization goals.

In addition to expanding our organization, we expect to increase the size of our facility and build out our development and manufacturing capabilities, which will require significant capital expenditures. If these capital

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expenditures are higher than expected, it may adversely affect our financial condition and capital resources. In addition, if the increase in the size of our facility is delayed, it may limit our ability to rapidly expand the size of our organization in order to meet our corporate goals.

If we fail to obtain additional financing, we may be unable to complete the development and commercialization of our product candidates.

Our operations have consumed substantial amounts of cash since inception. We expect to continue to spend substantial amounts to continue the clinical development of our product candidates, including our planned clinical development and preclinical studies of our product candidates and other programs. If approved, we will require significant additional amounts in order to launch and commercialize our product candidates.

As of September 30, 2014, we had cash and cash equivalents of approximately \$61.9 million. We estimate that our net proceeds from this offering will be approximately \$127.1 million, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us. We intend to use the net proceeds from this offering to fund (1) our ongoing and planned Phase 1/2 clinical trials of BPX-501; (2) pre-clinical studies for BPX-401, BPX-601 and BPX-701 and fund the Phase 1/2 clinical trial of BPX-401, partially fund the planned Phase 1/2 clinical trial of BPX-601 and fund the Phase 1/2 clinical trial of BPX-701 as well as preclinical development of our other CAR T and TCR programs; (3) our planned Phase 1/2 clinical trials of BPX-201 in combination with checkpoint inhibitors; (4) the construction of tenant improvements and the purchase of capital equipment at our Houston facility, to accommodate our anticipated personnel needs for the next three years and to support our planned in-house new product discovery and development, as well as process development and manufacturing for U.S. clinical trials of planned product candidates or certain critical components thereof; and (5) other working capital purposes, including general operating expenses. We believe that such proceeds together with our existing cash and cash equivalents will be sufficient to fund our operations through at least 2016. However, changing circumstances may cause us to consume capital significantly faster than we currently anticipate, and we may need to spend more money than currently expected because of circumstances beyond our control. We may require additional capital for the further development and commercialization of our product candidates and may need to raise additional funds sooner if we choose to expand more rapidly than we presently anticipate.

Additional funding may not be available on acceptable terms, or at all. We have no committed source of additional capital and if we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue the development or commercialization of our product candidates or other research and development initiatives. We could be required to seek collaborators for our product candidates at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available or relinquish or license on unfavorable terms our rights to our product candidates in markets where we otherwise would seek to pursue development or commercialization ourselves.

Any of the above events could significantly harm our business, prospects, financial condition and results of operations and cause the price of our common stock to decline.

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

We may seek additional capital through a combination of public and private equity offerings, debt financings, strategic partnerships and alliances and licensing arrangements. 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 may include liquidation or other preferences that adversely affect your rights as a stockholder. The incurrence of indebtedness

would result in increased fixed payment obligations and could involve certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. If we raise additional funds through strategic partnerships and alliances and licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies or product candidates, or grant licenses on terms unfavorable to us.

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We need to oversee manufacturing of a complex supply chain of cellular therapy product candidates, viral vectors and small molecule drugs.

Because of the complex nature of our products, we need to oversee manufacture of multiple components that require a diverse knowledge base and appropriate manufacturing personnel. The supply chain for these components is separate and distinct, and no single manufacturer can supply more than one component of each of our products. Additionally, it is likely that the cell therapy products will need to be made within an appropriate geographic location for the area in which the products will be utilized, so one cell therapy manufacturing facility may not be able to supply diverse geographic areas. Any lack of capabilities to store, freeze, thaw and infuse our cell therapies would adversely affect our business and prospects.

We expect to rely on third parties to manufacture a substantial portion of our clinical cell therapy product candidates, viral vectors and small molecule supplies in the United States and Europe.

We do not currently own any facility that may be used as our clinical-scale manufacturing and processing facility, and must currently rely on outside vendors to manufacture supplies and process our product candidates, which is and will need to be done on a patient-by-patient basis. We have not yet caused our product candidates to be manufactured or processed on a commercial scale. We may not be able to scale patient-by-patient manufacturing and processing to satisfy clinical or commercial demands for any of our product candidates. In addition, our anticipated reliance on a limited number of third-party manufacturers exposes us to the following risks:

- n We may be unable to identify manufacturers on acceptable terms or at all because the number of potential manufacturers is limited and the FDA or an equivalent foreign regulatory agency must approve any replacement contractor. This approval would require new testing and compliance inspections. In addition, a new manufacturer would have to be educated in, or develop substantially equivalent processes for, production of our products after receipt of FDA approval, if any.
- n Our third-party manufacturers might be unable to timely formulate and manufacture our product or produce the quantity and quality required to meet our clinical and commercial needs, if any.
- n Contract manufacturers may not be able to execute our manufacturing procedures appropriately.
- n Our future contract manufacturers may not perform as agreed or may not remain in the contract manufacturing business for the time required to supply our clinical trials or to successfully produce, store and distribute our products.
- n Manufacturers are subject to ongoing periodic unannounced inspection by the FDA, the Drug Enforcement Administration, or corresponding agencies in other geographic locations, to ensure strict compliance with cGMP and other government regulations and corresponding foreign standards. We do not have control over third-party manufacturers' compliance with these regulations and standards.

n We may not own, or may have to share, the intellectual property rights to any improvements made by our third-party manufacturers in the manufacturing process for our products.

n Our third-party manufacturers could breach or terminate their agreement with us. Each of these risks could delay our clinical trials, the approval, if any of our product candidates by the FDA or the commercialization of our product candidates or result in higher costs or deprive us of potential product revenue. In addition, we will rely on third parties to perform release tests on our product candidates prior to delivery to patients. If these tests are not appropriately done and test data are not reliable, patients could be put at risk of serious harm.

We expect to create our own manufacturing facility for supply of U.S. clinical and/or commercial cell therapy product candidate requirements, but there is no guarantee we will be able to do so.

Our intent to create internal manufacturing infrastructure will rely upon finding personnel with an appropriate background and training to staff and operate the facility on a daily basis. Should we be unable to find such individuals, we may need to rely on external contractors longer than anticipated, and train additional personnel to fill the needed roles. There are a small number of individuals with experience in cell therapy and the competition for such individuals is high.

Specifically, the establishment of a cell-therapy manufacturing facility is a complex endeavor requiring knowledgeable individuals who have successful previous experience in cleanroom designs. Cell therapy facilities, like other biological agent manufacturing facilities, require appropriate commissioning and validation activities to

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demonstrate that they operate as designed. Additionally, each manufacturing process must be validated through the performance of process validation runs to guarantee that the facility, personnel, equipment, and process work as designed. While we have developed our own manufacturing processes using an in-house process development team to maximize our understanding of our process, there is timing risk associated with in-house product manufacture.

Cell-based therapies rely on the availability of specialty raw materials, which may not be available to us on acceptable terms or at all.

Gene-modified cell therapy manufacture requires many specialty raw materials, some of which are manufactured by small companies with limited resources and experience to support a commercial product. Some suppliers typically support biomedical researchers or blood-based hospital businesses and may not have the capacity to support commercial products manufactured under cGMP by biopharmaceutical firms. The suppliers may be ill-equipped to support our needs, especially in non-routine circumstances like an FDA inspection or medical crisis, such as widespread contamination. We also do not have commercial supply arrangements with many of these suppliers, and may not be able to contract with them on acceptable terms or at all. Accordingly, we may experience delays in receiving key raw materials to support clinical or commercial manufacturing.

In addition, some raw materials are currently available from a single supplier, or a small number of suppliers. We cannot be sure that these suppliers will remain in business, or that they will not be purchased by one of our competitors or another company that is not interested in continuing to produce these materials for our intended purpose.

We currently have no marketing and sales organization and have no experience in marketing products. If we are unable to establish marketing and sales capabilities or enter into agreements with third parties to market and sell our product candidates, we may not be able to generate product revenue.

We currently have no sales, marketing or distribution capabilities and have no experience in marketing products. We intend to develop an in-house marketing organization and sales force, which will require significant capital expenditures, management resources and time. We will have to compete with other pharmaceutical and biotechnology companies to recruit, hire, train and retain marketing and sales personnel.

If we are unable or decide not to establish internal sales, marketing and distribution capabilities, we will pursue collaborative arrangements regarding the sales and marketing of our products, however, there can be no assurance that we will be able to establish or maintain such collaborative arrangements, or if we are able to do so, that they will have effective sales forces. Any revenue we receive will depend upon the efforts of such third parties, which may not be successful. We may have little or no control over the marketing and sales efforts of such third parties and our revenue from product sales may be lower than if we had commercialized our product candidates ourselves. We also face competition in our search for third parties to assist us with the sales and marketing efforts of our product candidates.

There can be no assurance that we will be able to develop in-house sales and distribution capabilities or establish or maintain relationships with third-party collaborators to commercialize any product in the United States or overseas.

A variety of risks associated with marketing our product candidates internationally could materially adversely affect our business.

We plan to seek regulatory approval of our product candidates outside of the United States and, accordingly, we expect that we will be subject to additional risks related to operating in foreign countries if we obtain the necessary approvals, including:

- n differing regulatory requirements in foreign countries;
- n unexpected changes in tariffs, trade barriers, price and exchange controls and other regulatory requirements;
- n economic weakness, including inflation, or political instability in particular foreign economies and markets;
- n compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- n foreign taxes, including withholding of payroll taxes;
- n foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country;

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- n difficulties staffing and managing foreign operations;
- n workforce uncertainty in countries where labor unrest is more common than in the United States;
- n potential liability under the Foreign Corrupt Practices Act of 1977 or comparable foreign regulations;
- n challenges enforcing our contractual and intellectual property rights, especially in those foreign countries that do not respect and protect intellectual property rights to the same extent as the United States;
- n production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- n business interruptions resulting from geo-political actions, including war and terrorism.

These and other risks associated with our international operations may materially adversely affect our ability to attain or maintain profitable operations.

We may form or seek strategic alliances or enter into additional licensing arrangements in the future, and we may not realize the benefits of such alliances or licensing arrangements.

We may form or seek strategic alliances, create joint ventures or collaborations or enter into additional licensing arrangements with third parties that we believe will complement or augment our development and commercialization efforts with respect to our product candidates and any future product candidates that we may develop. Any of these relationships may require us to incur non-recurring and other charges, increase our near and long-term expenditures, issue securities that dilute our existing stockholders or disrupt our management and business. In addition, we face significant competition in seeking appropriate strategic partners and the negotiation process is time-consuming and complex. Moreover, we may not be successful in our efforts to establish a strategic partnership or other alternative arrangements for our product candidates because they may be deemed to be at too early of a stage of development for collaborative effort and third parties may not view our product candidates as having the requisite potential to demonstrate safety and efficacy. If we license products or businesses, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate them with our existing operations and company culture. We cannot be certain that, following a strategic transaction or license, we will achieve the revenue or specific net income that justifies such transaction. Any delays in entering into new strategic partnership agreements related to our product candidates could delay the development and commercialization of our product candidates in certain geographies for certain indications, which would harm our business prospects, financial condition and results of operations.

Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

Because we rely on third parties to manufacture our drug substance and our drug product, and because we collaborate with various organizations and academic institutions on the advancement of our technology platform, we must, at times, share trade secrets with them. We seek to protect our proprietary technology in part by entering into confidentiality agreements and, if applicable, material transfer agreements, collaborative research agreements, consulting agreements or other similar agreements with our collaborators, advisors, employees and consultants prior

to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, such as trade secrets. Despite these contractual provisions, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's discovery of our trade secrets or other unauthorized use or disclosure would impair our competitive position and may have a material adverse effect on our business.

We may use our financial and human resources to pursue a particular research program or product candidate and fail to capitalize on programs or product candidates that may be more profitable or for which there is a greater likelihood of success.

Because we have limited resources, we may forego or delay pursuit of opportunities with certain programs or product candidates or for indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs for product candidates may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product

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candidate, we may relinquish valuable rights to that product candidate through strategic collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate, or we may allocate internal resources to a product candidate in a therapeutic area in which it would have been more advantageous to enter into a partnering arrangement. We are particularly susceptible to this risk because we are pursuing clinical and preclinical development program in each of our CaspaCIDE, DeCIDE, CIDE CAR and GoCAR-T technologies. Resources spent on one of these programs could result in fewer resources to further develop the other programs.

We have limited information available regarding the ultimate cost of our products, and cannot estimate what the cost of our products will be upon commercialization, should that occur.

We do not yet have sufficient information to reliably estimate the cost of the commercial manufacturing and processing of our product candidates, and the actual cost to manufacture and process our product candidates could materially and adversely affect the commercial viability of our product candidates. As a result, we may never be able to develop a commercially viable product. Because of the patient-specific nature of our manufacturing process, it is not amenable to traditional scale up to manufacture larger lots as is performed for traditional drugs and biological agents.

We and our contractors utilize hazardous materials in our business operations, and any claims relating to improper handling, storage, or disposal of these materials could harm our business.

Our activities involve the controlled use of potentially hazardous substances, including chemical and biological materials, by our third-party manufacturers. Our manufacturers are subject to federal, state and local laws and regulations in the United States governing the use, manufacture, storage, handling and disposal of medical and hazardous materials, and similar laws in other geographic regions. Although we believe that our manufacturers procedures for using, handling, storing and disposing of these materials comply with legally prescribed standards, we cannot completely eliminate the risk of contamination or injury resulting from medical or hazardous materials. As a result of any such contamination or injury, we may incur liability or local, city, state or federal authorities may curtail the use of these materials and interrupt our business operations. In the event of an accident, we could be held liable for damages or penalized with fines, and the liability could exceed our resources. We do not have any insurance for liabilities arising from medical or hazardous materials. Compliance with applicable environmental laws and regulations is expensive, and current or future environmental regulations may impair our research, development and production efforts, which could harm our business, prospects, financial condition or results of operations.

Our internal computer systems, or those used by our clinical investigators, contractors or consultants, may fail or suffer security breaches.

Despite the implementation of security measures, our internal computer systems and those of our contractors and consultants are vulnerable to damage from computer viruses and unauthorized access. While we have not experienced any such material system failure or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our development programs and our business operations. For example, the loss of clinical trial data from completed or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development and commercialization of our product candidates could be delayed.

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

Our operations, and those of our clinical investigators, contractors and consultants, could be subject to power shortages, telecommunications failures, water shortages, floods, earthquakes, hurricanes, typhoons, fires, extreme weather conditions, medical epidemics and other natural or man-made disasters or business interruptions, for which we are predominantly self-insured. The occurrence of any of these business disruptions could seriously harm our operations and financial condition and increase our costs and expenses. We rely on third-party manufacturers to produce and process our product candidates on a patient by patient basis. Our ability to obtain clinical supplies of our product candidates could be disrupted if the operations of these suppliers are affected by a man-made or natural disaster or other business interruption.

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Our employees, independent contractors, consultants, commercial partners and vendors may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.

We are exposed to the risk of employee fraud or other illegal activity by our employees, independent contractors, consultants, commercial partners and vendors. Misconduct by these parties could include intentional, reckless and/or negligent conduct that fails to: comply with the laws of the FDA and other similar foreign regulatory bodies; provide true, complete and accurate information to the FDA and other similar foreign regulatory bodies, comply with manufacturing standards we have established, comply with healthcare fraud and abuse laws in the United States and similar foreign fraudulent misconduct laws, or report financial information or data accurately or to disclose unauthorized activities to us. If we obtain FDA approval of any of our product candidates and begin commercializing those products in the United States, our potential exposure under such laws will increase significantly, and our costs associated with compliance with such laws are also likely to increase. These laws may impact, among other things, our current activities with principal investigators and research patients, as well as proposed and future sales, marketing and education programs. In particular, the promotion, sales and marketing of healthcare items and services, as well as certain business arrangements in the healthcare industry, are subject to extensive laws designed to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, structuring and commission(s), certain customer incentive programs and other business arrangements generally. Activities subject to these laws also involve the improper use of information obtained in the course of patient recruitment for clinical trials. The laws that may affect our ability to operate include, but are not limited to:

- n the federal Anti-Kickback Statute, which prohibits, among other things, knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe, or rebate), directly or indirectly, overtly or covertly, in cash or in kind, to induce, or in return for, either the referral of an individual, or the purchase, lease, order or recommendation of any good, facility, item or service for which payment may be made, in whole or in part, under a federal healthcare program, such as the Medicare and Medicaid programs;
- n federal civil and criminal false claims laws and civil monetary penalty laws, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid, or other third-party payors that are false or fraudulent or knowingly making a false statement to improperly avoid, decrease or conceal an obligation to pay money to the federal government;
- n the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created new federal criminal statutes that prohibit knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or obtain, by means of false or fraudulent pretenses, representations, or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, regardless of the payor (e.g., public or private) and knowingly and willfully falsifying, concealing or covering up by any trick or device a material fact or making any materially false statements in connection with the delivery of, or payment for, healthcare benefits, items or services relating to healthcare matters;

- n HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and their respective implementing regulations, which impose requirements on certain covered healthcare providers, health plans, and healthcare clearinghouses as well as their respective business associates that perform services for them that involve the use, or disclosure of, individually identifiable health information, relating to the privacy, security and transmission of individually identifiable health information without appropriate authorization;

- n the federal Physician Payment Sunshine Act, created under the Health Reform Law, and its implementing regulations, which requires manufacturers of drugs, devices, biologicals and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) to report annually to the United States Department of Health and Human Services, or HHS, information related to payments or other transfers of value made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members; and

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- n federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers.

Additionally, we are subject to state and foreign equivalents of each of the healthcare laws described above, among others, some of which may be broader in scope and may apply regardless of the payor.

Effective upon the completion of this offering, we will adopt a code of business conduct and ethics, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent inappropriate conduct may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. Efforts to ensure that our business arrangements will comply with applicable healthcare laws may involve substantial costs. It is possible that governmental and enforcement authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law interpreting applicable fraud and abuse or other healthcare laws and regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of civil, criminal and administrative penalties, damages, disgorgement, monetary fines, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, contractual damages, reputational harm, diminished profits and future earnings, and curtailment of our operations, any of which could adversely affect our ability to operate our business and our results of operations. In addition, the approval and commercialization of any of our product candidates outside the United States will also likely subject us to foreign equivalents of the healthcare laws mentioned above, among other foreign laws.

If product liability lawsuits are brought against us, we may incur substantial liabilities and may be required to limit commercialization of our product candidates.

We face an inherent risk of product liability as a result of the clinical testing of our product candidates and will face an even greater risk if we commercialize any products. For example, we may be sued if our product candidates cause or are perceived to cause injury or are found to be otherwise unsuitable during clinical testing, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability or a breach of warranties. Claims could also be asserted under state consumer protection acts. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit commercialization of our product candidates. Even successful defense would require significant financial and management resources. Regardless of the merits or eventual outcome, liability claims may result in:

- n decreased demand for our product candidates;
- n injury to our reputation;
- n withdrawal of clinical trial participants;
- n initiation of investigations by regulators;

- n costs to defend the related litigation;
- n a diversion of management's time and our resources;
- n substantial monetary awards to clinical trial participants or patients;
- n product recalls, withdrawals or labeling, marketing or promotional restrictions;
- n loss of revenue;
- n exhaustion of any available insurance and our capital resources;
- n the inability to commercialize any product candidate; and
- n a decline in our share price.

Our inability to obtain sufficient product liability insurance at an acceptable cost to protect against potential product liability claims could prevent or inhibit the commercialization of any products we develop, alone or with corporate collaborators. We currently carry \$10.0 million of product liability insurance covering our clinical trials. Although we maintain such insurance, our insurance policies may have various exclusions, and we may be subject to a product liability claim for which we have no coverage. We may have to pay any amounts awarded by a court or negotiated in a settlement that exceed our coverage limitations or that are not covered by our insurance, and we may not have, or be

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able to obtain, sufficient capital to pay such amounts. Even if our agreements with any future corporate collaborators entitle us to indemnification against losses, such indemnification may not be available or adequate should any claim arise.

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

Under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, if a corporation undergoes an ownership change (generally defined as a greater than 50% change (by value) in its equity ownership over a three-year period), the corporation's ability to use its pre-change net operating loss carryforwards and other pre-change tax attributes to offset its post-change income may be limited. As a result of our most recent private placements and other transactions that have occurred over the past three years, we may have experienced, and, upon completion of this offering, may experience, an ownership change. We may also experience ownership changes in the future as a result of subsequent shifts in our stock ownership. As of December 31, 2013, we had U.S. net operating loss carryforwards of approximately \$26.9 million, which begin to expire in 2024, and U.S. research and development credits of \$0.8 million, which could be limited if we experience an ownership change.

Risks Related to Government Regulation

The FDA regulatory approval process is lengthy and time-consuming, and we may experience significant delays in the clinical development and regulatory approval of our product candidates.

We have not previously submitted a Biologics License Application, or BLA, to the FDA, or similar approval filings to comparable foreign authorities. A BLA must include extensive preclinical and clinical data and supporting information to establish the product candidate's safety, purity and potency for each desired indication. The BLA must also include significant information regarding the chemistry, manufacturing and controls for the product. We expect the novel nature of our product candidates to create further challenges in obtaining regulatory approval. For example, the FDA has limited experience with commercial development of T cell therapies for cancer. In addition, the cell and gene therapy office of the FDA has limited experience with combination products that include a small molecule component. Approval of our product candidates, including BPX-501, will require this FDA office to consult with another division of the FDA, which may result in further challenges in obtaining regulatory approval, including in developing final product labeling. The regulatory approval pathway for our product candidates may be uncertain, complex, expensive and lengthy, and approval may not be obtained.

We may also experience delays in completing planned clinical trials for a variety of reasons, including delays related to:

- n the availability of financial resources to commence and complete our planned clinical trials;
- n reaching agreement on acceptable terms with prospective clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different clinical trial sites;
- n obtaining approval at each clinical trial site by an independent institutional review board, or IRB;

- n recruiting suitable patients to participate in a clinical trial;
- n having patients complete a clinical trial or return for post-treatment follow-up;
- n clinical trial sites deviating from clinical trial protocol, failing to follow GCPs, or dropping out of a clinical trial;
- n adding new clinical trial sites; or
- n manufacturing sufficient quantities of qualified materials under cGMPs and applying them on a subject by subject basis for use in clinical trials.

We could also encounter delays if physicians encounter unresolved ethical issues associated with enrolling patients in clinical trials of our product candidates in lieu of prescribing existing treatments that have established safety and efficacy profiles. Further, a clinical trial may be suspended or terminated by us, the IRBs for the institutions in which such clinical trials are being conducted, the Data Monitoring Committee for such clinical trial, or by the FDA or other regulatory authorities 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 clinical 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 product candidate, changes in governmental

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regulations or administrative actions or lack of adequate funding to continue the clinical trial. If we experience termination of, or delays in the completion of, any clinical trial of our product candidates, the commercial prospects for our product candidates will be harmed, and our ability to generate product revenue will be delayed. In addition, any delays in completing our clinical trials will increase our costs, slow down our product development and approval process and jeopardize our ability to commence product sales and generate revenue.

Many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may ultimately lead to the denial of regulatory approval of our product candidates.

The FDA may disagree with our regulatory plans and we may fail to obtain regulatory approval of our product candidates.

Our ongoing and planned Phase 1 and Phase 1/2 clinical trials of BPX-501 are designed to show enhanced immune system recovery in patients following an allogeneic (donor cells as opposed to the patient's own cells) HSCT. Following the completion of those clinical trials, and if the results are satisfactory, we plan to meet with the FDA in an end of phase two meeting to discuss our clinical trial design that could serve as the registration trial for our BLA for BPX-501 in that indication. We, or our institutional collaborators, are conducting and planning additional Phase 1 and Phase 1/2 clinical trials of BPX-501 in clinical trials designed to evaluate BPX-501 as a treatment for patients with recurrent disease (relapse) after an allogeneic HSCT. Following the completion of those clinical trials, and if the results are satisfactory, we plan to meet with the FDA in another end of phase two meeting to discuss whether our planned clinical trial design could serve as the registration trial for our BLA for BPX-501 in that indication. However, the general approach for FDA approval of a new biologic or drug is dispositive data from two adequate and well-controlled, Phase 3 clinical trials of the relevant biologic or drug in the relevant patient population. Phase 3 clinical trials typically involve hundreds of patients, have significant costs and take years to complete. We believe that a single Phase 3 clinical trial strategy is warranted given the limited alternatives for patients for which BPX-501 therapy is potentially beneficial, but the FDA may ultimately require more than one Phase 3 clinical trial and may limit clinical trial designs allowed to serve as a registration trial.

Our clinical trials results may not support approval. In addition, BPX-501 and our other product candidates could fail to receive regulatory approval for many reasons, including the following:

- n the FDA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials;
- n we may be unable to demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities that our product candidates have the necessary safety, purity, and potency for any of their proposed indications;
- n the results of clinical trials may not meet the level of statistical significance required by the FDA or comparable foreign regulatory authorities for approval;
- n we may be unable to demonstrate that our product candidates' clinical and other benefits outweigh their safety risks;

- n we may encounter serious and unexpected adverse events during clinical trials that render our products unsafe for use in humans;
- n the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from preclinical studies or clinical trials;
- n the data collected from clinical trials of our product candidates may not be sufficient to the satisfaction of the FDA or comparable foreign regulatory authorities to support the submission of a BLA or other comparable submission in foreign jurisdictions or to obtain regulatory approval in the United States or elsewhere;
- n the FDA or comparable foreign regulatory authorities may fail to approve our manufacturing processes and/or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; and
- n the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

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Obtaining and maintaining regulatory approval of our product candidates in one jurisdiction does not mean that we will be successful in obtaining regulatory approval of our product candidates in other jurisdictions.

Obtaining and maintaining regulatory approval of our product candidates in one jurisdiction does not guarantee that we will be able to obtain or maintain regulatory approval in any other jurisdiction, while a failure or delay in obtaining regulatory approval in one jurisdiction may have a negative effect on the regulatory approval process in others. For example, even if the FDA grants marketing approval of a product candidate, comparable regulatory authorities in foreign jurisdictions must also approve the manufacturing, marketing and promotion of the product candidate in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and greater than, those in the United States, including additional preclinical studies or clinical trials. Studies and clinical trials conducted in one jurisdiction may not be accepted by regulatory authorities in other jurisdictions. In many jurisdictions outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that jurisdiction. In some cases, the price that we intend to charge for our products is also subject to approval.

We may also submit marketing applications in other countries. Regulatory authorities in jurisdictions outside of the United States have requirements for approval of product candidates with which we must comply prior to marketing in those jurisdictions. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our products in certain countries. If we fail to comply with the regulatory requirements in international markets and/or receive applicable marketing approvals, our target market will be reduced and our ability to realize the full market potential of our product candidates will be harmed.

Even if we receive regulatory approval of our product candidates, we will be subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense and we may be subject to penalties and/or withdrawal of product approval if we fail to comply with regulatory requirements or experience unanticipated problems with our product candidates.

Any regulatory approvals that we receive for our product candidates will require surveillance to monitor the safety and efficacy of the product candidate. The FDA may also require a risk evaluation and mitigation strategy, or REMS, in order to approve our product candidates, which could entail requirements for a medication guide, physician communication plans or additional elements to ensure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. In addition, if the FDA or a comparable foreign regulatory authority approves our product candidates, the manufacturing processes, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion, import, export and recordkeeping for our product candidates will be subject to extensive and ongoing regulatory requirements. These requirements include, among other things, submissions of safety and other post-marketing information and reports, registration, as well as continued compliance with cGMPs and cGCPs for any clinical trials that we conduct post-approval. Later discovery of previously unknown problems with our product candidates, including adverse events of unanticipated severity or frequency, or our or with our third-party manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may result in, among other things:

- n restrictions on the marketing or manufacturing of our product candidates, withdrawal of the product from the market, or voluntary or mandatory product recalls;

- n fines, warning letters or holds on clinical trials;

- n refusal by the FDA to approve pending applications or supplements to approved applications filed by us or suspension or revocation of license approvals;

- n suspension or termination of manufacturing at one or more manufacturing facilities;

- n product seizure or detention, or refusal to permit the import or export of our product candidates; and

- n injunctions or the imposition of civil or criminal penalties.

The FDA's and other regulatory authorities' policies may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability.

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Even if we obtain regulatory approval of our product candidates, the products may not gain market acceptance among physicians, patients, hospitals, cancer treatment centers, third-party payors and others in the medical community.

The use of engineered T cells as a potential cancer treatment is a recent development and may not become broadly accepted by physicians, patients, hospitals, cancer treatment centers, third-party payors and others in the medical community. We expect physicians in the large bone marrow transplant centers to be particularly influential and we may not be able to convince them to use our product candidates for many reasons. Factors will influence whether our product candidates are accepted in the market, including:

- n the clinical indications for which our product candidates are approved;
- n physicians, hospitals, cancer treatment centers and patients considering our product candidates as a safe and effective treatment;
- n the potential and perceived advantages of our product candidates over alternative treatments;
- n the prevalence and severity of any side effects;
- n product labeling or product insert requirements of the FDA or other regulatory authorities;
- n limitations or warnings contained in the labeling approved by the FDA;
- n the timing of market introduction of our product candidates as well as competitive products;
- n the cost of treatment in relation to alternative treatments;
- n the availability of adequate coverage, reimbursement and pricing by third-party payors and government authorities;
- n the willingness of patients to pay out-of-pocket in the absence of coverage by third-party payors, including government authorities;
- n relative convenience and ease of administration, including as compared to alternative treatments and competitive therapies;

n confusion or lack of understanding regarding the effects of rimiducid and the timing and size of dosing of rimiducid after immune cell therapy; and

n the effectiveness of our sales and marketing efforts.

In addition, although we are not utilizing embryonic stem cells or replication competent vectors, adverse publicity due to the ethical and social controversies surrounding the therapeutic use of such technologies, and reported side effects from any clinical trials using these technologies or the failure of such clinical trials to demonstrate that these therapies are safe and effective may limit market acceptance our product candidates. If our product candidates are approved but fail to achieve market acceptance among physicians, patients, hospitals, cancer treatment centers or others in the medical community, we will not be able to generate significant revenue.

Even if our products achieve market acceptance, we may not be able to maintain that market acceptance over time if new products or technologies are introduced that are more favorably received than our products, are more cost effective or render our products obsolete.

We plan to seek orphan drug designation for BPX-501 and rimiducid as a combination therapy, but we may be unable to obtain such designation or maintain the benefits associated with orphan drug status, including market exclusivity.

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug or biologic intended to treat a rare disease or condition or for which there is no reasonable expectation that the cost of developing and making available in the United States a drug or biologic for a disease or condition will be recovered from sales in the United States for that drug or biologic. If a product that has orphan drug designation subsequently receives the first FDA approval for the disease for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications, including a BLA to market the same biologic for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity.

Rimiducid has orphan drug designation for the treatment of acute graft-versus-host-disease, or GvHD, in patients undergoing bone marrow transplantation. Since BPX-501 and rimiducid are considered a combination product, we

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are currently discussing the designation of orphan for the combination of BPX-501 and rimiducid for treatment of immunodeficiency after allogeneic transplant with the FDA, but exclusive marketing rights in the United States may be limited if we seek approval for an indication broader than the orphan designated indication and may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition. In addition, while we intend to seek orphan drug designation for other product candidates, we may never receive such designations.

Coverage and reimbursement may be limited or unavailable in certain market segments for our product candidates, which could make it difficult for us to sell our product candidates profitably.

Successful sales of our product candidates, if approved, depend on the availability of coverage and adequate reimbursement from third-party payors. In addition, because our product candidates represent new approaches to the treatment of cancer, we cannot accurately estimate the potential revenue from our product candidates.

Patients who are provided medical treatment for their conditions generally rely on third-party payors to reimburse all or part of the costs associated with their treatment. Coverage and adequate reimbursement from governmental healthcare programs, such as Medicare and Medicaid, and commercial payors is critical to new product acceptance.

Government authorities and other third-party payors, such as private health insurers and health maintenance organizations, decide which drugs and treatments they will cover and the amount of reimbursement. Reimbursement by a third-party payor may depend upon a number of factors, including, but not limited to, the third-party payor's determination that use of a product is:

- n a covered benefit under its health plan;
- n safe, effective and medically necessary;
- n appropriate for the specific patient;
- n cost-effective; and

- n neither experimental nor investigational.

Obtaining coverage and reimbursement approval of a product from a government or other third-party payor is a time-consuming and costly process that could require us to provide to the payor supporting scientific, clinical and cost-effectiveness data for the use of our products. Even if we obtain coverage for a given product, the resulting reimbursement levels might not be adequate for us to achieve or sustain profitability or may require co-payments that patients find unacceptably high. Patients are unlikely to use our product candidates unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of our product candidates.

In the United States, no uniform policy of coverage and reimbursement for products exists among third-party payors. Therefore, coverage and reimbursement for products can differ significantly from payor to payor. As a result, the

coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our product candidates to each payor separately, with no assurance that coverage and adequate reimbursement will be obtained.

We intend to seek approval to market our product candidates in both the United States and in selected foreign jurisdictions. If we obtain approval in one or more foreign jurisdictions for our product candidates, we will be subject to rules and regulations in those jurisdictions. In some foreign countries, particularly those in the EU, the pricing of biologics is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after obtaining marketing approval of a product candidate. In addition, market acceptance and sales of our product candidates will depend significantly on the availability of coverage and adequate reimbursement from third-party payors for our product candidates and may be affected by existing and future health care reform measures.

Third-party payors, whether domestic or foreign, or governmental or commercial, are developing increasingly sophisticated methods of controlling healthcare costs. In both the United States and certain foreign jurisdictions, there have been a number of legislative and regulatory changes to the health care system that could impact our ability to sell our products profitably. In particular, in 2010, the Patient Protection and Affordable Care Act, as

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amended by the Health Care and Education Reconciliation Act, collectively, the Healthcare Reform Act, was enacted in the United States. The Healthcare Reform Act and its implementing regulations, among other things, revised the methodology by which rebates owed by manufacturers to the state and federal government for covered outpatient drugs and certain biologics, including our product candidates, under the Medicaid Drug Rebate Program are calculated, increased the minimum Medicaid rebates owed by most manufacturers under the Medicaid Drug Rebate Program, extended the Medicaid Drug Rebate program to utilization of prescriptions of individuals enrolled in Medicaid managed care organizations, subjected manufacturers to new annual fees and taxes for certain branded prescription drugs, and provided incentives to programs that increase the federal government's comparative effectiveness research.

Other legislative changes have been proposed and adopted in the United States since the Healthcare Reform Act was enacted. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least \$1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions of Medicare payments to providers up to 2% per fiscal year, which went into effect on April 1, 2013, and will stay in effect through 2024 unless Congressional action is taken. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, or the ATRA, which, among other things, reduced Medicare payments to several providers, including hospitals, imaging centers and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

There have been, and likely will continue to be, legislative and regulatory proposals at the foreign, federal and state levels directed at broadening the availability of healthcare and containing or lowering the cost of healthcare. We cannot predict the initiatives that may be adopted in the future. The continuing efforts of the government, insurance companies, managed care organizations and other payors of healthcare services to contain or reduce costs of healthcare and/or impose price controls may adversely affect:

- n the demand for our product candidates, if we obtain regulatory approval;
- n our ability to set a price that we believe is fair for our products;
- n our ability to generate revenue and achieve or maintain profitability;
- n the level of taxes that we are required to pay; and
- n the availability of capital.

Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors, which may adversely affect our future profitability.

Third party payors may also have difficulty in determining the appropriate coverage of our product candidates, if approved, due to the fact that they are combination products that include a small molecule drug (rimiducid). To the extent there are any delays in determining such coverage or inadequate coverage for all aspects of our combination

therapies, it would adversely affect the market acceptance of our product candidates.

Due to the novel nature of our technology and the small size of our target patient populations, we face uncertainty related to pricing and reimbursement for these product candidates.

Our target patient populations are relatively small, as a result, the pricing and reimbursement of our product candidates, if approved, must be adequate to support commercial infrastructure. If we are unable to obtain adequate levels of reimbursement, our ability to successfully market and sell our product candidates will be adversely affected. The manner and level at which reimbursement is provided for services related to our product candidates (e.g., for administration of our product to patients) is also important. Inadequate reimbursement for such services may lead to physician resistance and adversely affect our ability to market or sell our products.

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Risks Related to Our Intellectual Property

We depend on intellectual property licensed from third parties and termination of any of these licenses could result in the loss of significant rights, which would harm our business.

We are dependent on patents, know-how and proprietary technology, both our own and licensed from others. Technology that we license from others includes rimiducid, which is the small molecule activating agent that forms a part of our current and future product candidates and that we license from ARIAD. ARIAD may terminate or modify our license upon a material breach by us that remains uncured following the date that is 30 days after written notice of a payment breach and 90 days for any other breach, and effective immediately upon certain insolvency events. In addition, ARIAD may terminate our license, upon notice to us, if we do not make certain lump sum installment payments within specific timeframes from the date such payments are due. In addition, ARIAD in-licenses some of the intellectual property rights it licenses to us. To the extent ARIAD fails to meet its obligations under its license agreements, which we are not in control of, we may lose the benefits of our license agreement with ARIAD. We license from Baylor College of Medicine, or Baylor, certain intellectual property related to methods for activating antigen presenting cells and to certain genetic constructs. Baylor may terminate or modify our licenses in the event of a material breach by us that remains uncured following the date that is 90 days after written notice of such breach or upon certain insolvency events that remain uncured following the date that is 30 days following written notice of such insolvency event. In addition, we have funded certain of our ongoing clinical development and will fund certain of our future clinical development with funds from the state of Texas. The state may have rights to commercialize the results of those clinical trials if it determines that we have failed, after notice and an opportunity to cure, to use diligent and commercially reasonable efforts to commercialize or otherwise bring to practical application the results of the funded clinical trials. We are in advanced negotiations with Baylor for an exclusive license to intellectual property concerning the use of inducible caspase technology for elimination of transplanted cells.

Any termination of these agreements could result in the loss of significant rights and could harm our ability to commercialize our product candidates. See [Business Our License Agreements](#) for additional information regarding our license agreements.

Disputes may also arise between us and our licensors and other partners regarding intellectual property subject to a license agreement, including:

- n the scope of rights granted under the license agreement and other interpretation-related issues;
- n whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- n our right to sublicense patent and other rights to third parties under collaborative development relationships;
- n our diligence obligations with respect to the use of the licensed technology in relation to our development and commercialization of our product candidates, and what activities satisfy those diligence obligations; and

n the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners.

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates.

We are generally also subject to all of the same risks with respect to protection of intellectual property that we license, as we are for intellectual property that we own, which are described below. If we or our licensors fail to adequately protect this intellectual property, our ability to commercialize products could suffer.

If our efforts to protect the proprietary nature of our technologies are not adequate, we may not be able to compete effectively in our market.

Any disclosure to or misappropriation by third parties of our confidential proprietary information could enable competitors to quickly duplicate or surpass our technological achievements, thus eroding our competitive position in our market. Certain intellectual property which is covered by our in-license agreements has been developed at academic institutions which have retained non-commercial rights to such intellectual property.

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There are several pending U.S. and foreign patent applications in our portfolio, and we anticipate additional patent applications will be filed both in the United States and in other countries, as appropriate. However, we cannot predict:

- n if and when patents will issue;

- n the degree and range of protection any issued patents will afford us against competitors including whether third parties will find ways to invalidate or otherwise circumvent our patents;

- n whether or not others will obtain patents claiming aspects similar to those covered by our patents and patent applications; or

- n whether we will need to initiate litigation or administrative proceedings which may be costly whether we win or lose.

Composition of matter patents for biological and pharmaceutical products are generally considered to be the strongest form of intellectual property. We cannot be certain that the claims in our pending patent applications directed to compositions of matter for our product candidates will be considered patentable by the United States Patent and Trademark Office, or the USPTO, or by patent offices in foreign countries, or that the claims in any of our issued patents will be considered valid by courts in the United States or foreign countries. Method of use patents have claims directed to the use of a product for the specified method. This type of patent does not prevent a competitor from making and marketing a product that is identical to our product for an indication that is outside the scope of the patented method. Moreover, even if competitors do not actively promote their product for our targeted indications, physicians may prescribe these products off-label. Although off-label prescriptions may infringe or contribute to the infringement of method of use patents, the practice is common and such infringement is difficult to prevent or prosecute.

The strength of patents in the biotechnology and pharmaceutical field involves complex legal and scientific questions and can be uncertain. The patent applications that we own or in-license may fail to result in issued patents with claims that cover our product candidates or uses thereof in the United States or in other foreign countries. Even if the patents do successfully issue, third parties may challenge the validity, enforceability or scope thereof, which may result in such patents being narrowed, invalidated or held unenforceable. Furthermore, even if they are unchallenged, patents in our portfolio may not adequately exclude third parties from practicing relevant technology or prevent others from designing around our claims. If the breadth or strength of our intellectual property position with respect to our product candidates is threatened, it could dissuade companies from collaborating with us to develop, and threaten our ability to commercialize, our product candidates. Further, if we encounter delays in our clinical trials, the period of time during which we could market our product candidates under patent protection would be reduced. Since patent applications in the United States and most other countries are confidential for a period of time after filing, we cannot be certain that patent applications in our portfolio were the first filed patent applications related to our product candidates. Furthermore, for United States applications in which all claims are entitled to a priority date before March 16, 2013, an interference proceeding can be provoked by a third-party or instituted by the USPTO, to determine who was the first to invent any of the subject matter covered by the patent claims of our applications. For United States applications containing a claim not entitled to priority before March 16, 2013, there is a greater level of uncertainty in the patent law with the passage of the America Invents Act (2012) which brings into effect significant changes to the U.S. patent laws that are yet untried and untested, and which introduces new procedures for challenging pending

patent applications and issued patents. A primary change under this reform is creating a first to file system in the U.S. This will require us to be cognizant going forward of the time from invention to filing of a patent application.

We are aware that patent coverage on rimiducid, the dimerization molecule AP1903, will expire in 2016. Any additional barriers to entry for competitors to use rimiducid after patent expiration may not be effective in preventing such use. There remain significant questions regarding how the FDA will interpret the biosimilar provisions recently added to the Public Health Service Act as applied to complex biological products such as our investigational products. Depending on how the FDA ultimately interprets these provisions, if our investigational products incorporating rimiducid receive FDA approval through a combination product BLA, then a biosimilar of these combination products could be approved by the FDA 12 years from the date that we receive FDA approval for our application. In addition, if a third party were able to obtain FDA approval of a new drug application for rimiducid on

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its own, then it is possible that other third parties could later seek approval of an abbreviated new drug application for rimiducid.

We rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable, processes for which patents are difficult to enforce and any other elements of our product discovery and development processes that involve proprietary know-how, information, or technology that is not covered by patents. We require all of our employees to assign their inventions to us, and require all of our employees, consultants, advisors and any third parties who have access to our proprietary know-how, information, or technology to enter into confidentiality agreements; however, we cannot be certain that our trade secrets and other confidential proprietary information will not be disclosed or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. Furthermore, the laws of some foreign countries do not protect proprietary rights to the same extent or in the same manner as the laws of the United States. As a result, we may encounter significant problems in protecting and defending our intellectual property both in the United States and abroad. If we are unable to prevent unauthorized material disclosure of our intellectual property to third parties, we will not be able to establish or maintain a competitive advantage in our market, which could materially adversely affect our business, operating results and financial condition.

Third-party claims of intellectual property infringement may prevent or delay our product discovery and development efforts.

Our commercial success depends in part on our avoiding infringement of the patents and proprietary rights of third parties. There is a substantial amount of litigation involving patents and other intellectual property rights in the biotechnology and pharmaceutical industries, as well as administrative proceedings for challenging patents, including interference and reexamination proceedings before the USPTO or oppositions and other comparable proceedings in foreign jurisdictions. Recently, under U.S. patent reform, new procedures including *inter partes* review and post grant review have been implemented. As stated above, this reform is untried and untested and will bring uncertainty to the possibility of challenge to our patents in the future. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are developing our product candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may give rise to claims of infringement of the patent rights of others.

Third parties may assert that we are employing their proprietary technology without authorization. There may be third-party patents, of which we are currently unaware or have not sufficiently analyzed with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that our product candidates may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. If any third-party patents were held by a court of competent jurisdiction to cover aspects of our formulations, the manufacturing process of our product candidates, constructs or molecules used in or formed during the manufacturing process, methods of use, including combination therapy or patient selection methods or any final product itself, the holders of any such patents may be able to block our ability to develop and commercialize the product candidate unless we obtained a license under the applicable patents, or until such patents expire or they are finally determined to be held invalid or unenforceable. In either case, such a license may not be available on commercially reasonable terms or at all. If we are unable to obtain a necessary license to a third-party patent on commercially reasonable terms, or at all, our ability to commercialize our product candidates may be impaired or delayed, which could in turn significantly harm our business.

Parties making claims against us may seek and obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, obtain one or more licenses from third parties, pay royalties or redesign our infringing products, which may be impossible or require substantial time and monetary expenditure. We cannot predict whether any such license would be available at all or whether it would be available on commercially reasonable terms. Furthermore, even in the absence of litigation, we may need to obtain licenses from third parties to advance our research or allow commercialization of our product candidates. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we would

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be unable to further develop and commercialize our product candidates, which could harm our business significantly.

We are aware of a third party patent having claims directed to chimeric DNA comprising DNA segments encoding (1) a single chain antibody domain and (2) transmembrane and cytoplasmic domains of an endogenous protein. Even though we have reason to believe that our BPX-401 and BPX-601 technologies are not covered by claims of this patent, an owner or licensee of the patent still might bring a patent infringement suit against us. If the patent is asserted against us, we may not prevail in defending against claims of infringement and/or challenging the validity of claims in the patent. We may not successfully develop alternative technologies or enter into an agreement by which we obtain rights to the patent. These rights, if necessary, may not be available on terms acceptable to us.

Also, while we are aware there are third party patents having claims that may be considered relevant to BPX-201, BPX-401 and BPX-601 technologies for which we are seeking, or plan to seek, regulatory approval, we believe those patents have a patent term that may expire prior to the time we expect to obtain regulatory approval for these technologies. The estimated expiration dates for those patents were determined according to information on the face pages of the patents, and certain factors that could influence patent term, such as patent term adjustment and patent term extension, for example, were not factored into these estimates. Accordingly, the estimated expiration dates of those patents may not be accurate and one or more of those patents may not expire before we obtain regulatory approval for an applicable technology. Owners or licensees of one or more of those patents may bring a patent infringement suit against us. If one or more of those patents are asserted against us, we may be able to assert a defense for a safe harbor to patent infringement under 35 U.S.C. 271(e)(1) if certain requirements are met. It is possible that (1) certain of these requirements may not be met, and/or (2) one or more of the third party patents might expire after one or more of our technologies obtain regulatory approval, and consequently we may not successfully assert such a defense to patent infringement. If we are unsuccessful in asserting a defense under 35 U.S.C. 271(e)(1), it is possible we may not prevail in defending against claims of infringement and/or challenging the validity of claims in those patents. We may not successfully develop alternative technologies or enter into agreements by which we obtain rights to applicable patents. These rights, if necessary, may not be available on terms acceptable to us.

We are also aware of third party patents having claims directed to single-chain antibody fragments that bind to prostate stem cell antigen, or PSCA, and those patents may be considered relevant to BPX-601 technologies we are developing. We are currently evaluating whether or not a license may be obtained for rights to those patents. If we determine it is necessary to obtain rights to one or more of those patents, we may not successfully enter into an agreement or agreements required for obtaining rights to those patents, and these rights may not be available on terms acceptable to us. We also may not successfully develop alternative technologies if we cannot secure rights to those patents.

We may not be successful in obtaining or maintaining necessary rights to product components and processes for our development pipeline through acquisitions and in-licenses.

Because our programs may involve additional product candidates that may require the use of proprietary rights held by third parties, the growth of our business will likely depend in part on our ability to acquire, in-license or use these proprietary rights.

Our product candidates may also require specific formulations to work effectively and efficiently and these rights may be held by others. We may be unable to acquire or in-license any compositions, methods of use, processes or other third-party intellectual property rights from third parties that we identify. Even if we are able to obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In that event, we may be required to expend significant time and resources to develop or license replacement technology.

The licensing and acquisition of third-party intellectual property rights is a competitive area, and companies, which may be more established, or have greater resources than we do, may also be pursuing strategies to license or acquire third-party intellectual property rights that we may consider necessary or attractive in order to commercialize our product candidates. More established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities.

There can be no assurance that we will be able to successfully complete negotiations and ultimately acquire the rights to the intellectual property that we may seek to acquire in the future.

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We may be involved in lawsuits to protect or enforce our patents or the patents of our licensors, which could be expensive, time-consuming and unsuccessful.

Competitors may infringe our patents or the patents of our licensors. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that one or more of our patents is not valid or is unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated, held unenforceable, or interpreted narrowly and could put our patent applications at risk of not issuing. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, obtain one or more licenses from third parties, pay royalties or redesign our infringing products, which may be impossible or require substantial time and monetary expenditure.

Interference proceedings provoked by third parties or brought by the USPTO may be necessary to determine the priority of inventions with respect to our patents or patent applications or those of our licensors. An unfavorable outcome could result in a loss of our current patent rights and could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Litigation or interference proceedings may result in a decision adverse to our interests and, even if we are successful, may result in substantial costs and distract our management and other employees. We may not be able to prevent, alone or with our licensors, misappropriation of our trade secrets or confidential information, particularly in countries where the laws may not protect those rights as fully as in the United States.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock.

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

Periodic maintenance fees on any issued patent are due to be paid to the USPTO and foreign patent agencies in several stages over the lifetime of the patent. The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Noncompliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. Such noncompliance events are outside of our direct control for (1) non-U.S. patents and patent applications owned by us, and (2) patents and patent applications licensed to us by another entity. In such an event, our competitors might be able to enter the market, which would have a material adverse effect on our business.

Issued patents covering our product candidates could be found invalid or unenforceable if challenged in court or the USPTO.

If we or one of our licensing partners initiate legal proceedings against a third party to enforce a patent covering one of our product candidates, the defendant could counterclaim that the patent covering our product candidate, as applicable, is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace, and there are numerous grounds upon which a third party can assert invalidity or unenforceability of a patent. Third parties may also raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, post grant review, and equivalent proceedings in foreign jurisdictions (e.g., opposition

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proceedings). Such proceedings could result in revocation or amendment to our patents in such a way that they no longer cover our product candidates. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art and that prior art that was cited during prosecution, but not relied on by the patent examiner, will not be revisited. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we would lose at least part, and perhaps all, of the patents directed to our product candidates. Such a loss of patent rights could have a material adverse impact on our business.

Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our products.

As is the case with other biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involve both technological and legal complexity, and is therefore costly, time-consuming and inherently uncertain. In addition, the United States has recently enacted and is currently implementing wide-ranging patent reform legislation. Recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future. For example, in the recent case, *Assoc. for Molecular Pathology v. Myriad Genetics, Inc.*, the U.S. Supreme Court held that certain claims to DNA molecules are not patentable. While we do not believe that any of the patents owned or licensed by us will be found invalid based on this decision, we cannot predict how future decisions by the courts, the U.S. Congress or the USPTO may impact the value of our patents.

We have limited foreign intellectual property rights and may not be able to protect our intellectual property rights throughout the world.

We have limited intellectual property rights outside the United States. Filing, prosecuting and defending patents on product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patents to develop their own products and further, may export otherwise infringing products to territories where we have patents, but enforcement is not as strong as that in the United States. These products may compete with our products and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property in foreign jurisdictions. The legal systems of certain countries, particularly China and certain other developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property, particularly those relating to biopharmaceutical products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. To date, we have not sought to enforce any issued patents in these foreign jurisdictions. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents

at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. The requirements for patentability may differ in certain countries, particularly developing countries. Furthermore, generic drug manufacturers or other competitors may challenge the scope, validity or enforceability of our or our licensors' patents, requiring us or our licensors to engage in complex, lengthy and costly litigation or other proceedings. Certain countries in Europe and developing countries, including China and India, have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In those countries, we and our licensors may have limited remedies if patents are infringed or if we or our licensors are compelled to grant a license to a third party, which could materially

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diminish the value of those patents. This could limit our potential revenue opportunities. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of third parties.

We have received confidential and proprietary information from third parties. In addition, we employ individuals who were previously employed at other biotechnology or pharmaceutical companies. We may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed confidential information of these third parties or our employees' former employers. Litigation may be necessary to defend against these claims. Even if we are successful in defending against these claims, litigation could result in substantial cost and be a distraction to our management and employees.

Risks Related to This Offering and Ownership of our Common Stock

We do not know whether an active, liquid and orderly trading market will develop for our common stock or what the market price of our common stock will be and as a result it may be difficult for you to sell your shares of our common stock.

Prior to this offering there has been no public market for shares of our common stock. Although we have applied to list our common stock on The NASDAQ Global Market, an active trading market for our shares may never develop or be sustained following this offering. You may not be able to sell your shares quickly or at the market price if trading in shares of our common stock is not active. The initial public offering price for our common stock was determined through negotiations with the underwriters, and the negotiated price may not be indicative of the market price of the common stock after the offering. As a result of these and other factors, you may be unable to resell your shares of our common stock at or above the initial public offering price. Further, an inactive market may also impair our ability to raise capital by selling shares of our common stock and may impair our ability to enter into strategic partnerships or acquire companies or products by using our shares of common stock as consideration.

The price of our stock may be volatile, and you could lose all or part of your investment.

The trading price of our common stock following this offering is likely to be highly volatile and could be subject to wide fluctuations in response to various factors, some of which are beyond our control, including limited trading volume. In addition to the factors discussed in this Risk Factors section and elsewhere in this prospectus, these factors include:

- n the commencement, enrollment or results of the planned clinical trials of our product candidates or any future clinical trials we may conduct, or changes in the development status of our product candidates;
- n any delay in our regulatory filings for our product candidates and any adverse development or perceived adverse development with respect to the applicable regulatory authority's review of such filings, including without limitation the FDA's issuance of a refusal to file letter or a request for additional information;

- n adverse results or delays in clinical trials;
- n our decision to initiate a clinical trial, not to initiate a clinical trial or to terminate an existing clinical trial;
- n adverse regulatory decisions, including failure to receive regulatory approval of our product candidates;
- n changes in laws or regulations applicable to our products, including but not limited to clinical trial requirements for approvals;
- n adverse developments concerning our CID technology platform and our small molecule drug rimiducid;
- n adverse developments concerning our manufacturers;
- n our inability to obtain adequate product supply for any approved product or inability to do so at acceptable prices;
- n our inability to establish collaborations if needed;
- n our failure to commercialize our product candidates;
- n additions or departures of key scientific or management personnel;
- n unanticipated serious safety concerns related to the use of our product candidates;
- n introduction of new products or services offered by us or our competitors;

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- n announcements of significant acquisitions, strategic partnerships, joint ventures or capital commitments by us or our competitors;
- n our ability to effectively manage our growth;
- n the size and growth of our initial target markets;
- n our ability to successfully treat additional types of diseases and cancers or at different stages;
- n actual or anticipated variations in quarterly operating results;
- n our cash position;
- n our failure to meet the estimates and projections of the investment community or that we may otherwise provide to the public;
- n publication of research reports about us or our industry, or immunotherapy in particular, or positive or negative recommendations or withdrawal of research coverage by securities analysts;
- n changes in the market valuations of similar companies;
- n overall performance of the equity markets;
- n sales of our common stock by us or our stockholders in the future;
- n trading volume of our common stock;
- n changes in accounting practices;
- n ineffectiveness of our internal controls;
- n disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;

n significant lawsuits, including patent or stockholder litigation;

n general political and economic conditions; and

n other events or factors, many of which are beyond our control.

In addition, the stock market in general, and The NASDAQ Global Market and biopharmaceutical companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance. If the market price of our common stock after this offering does not exceed the initial public offering price, you may not realize any return on your investment in us and may lose some or all of your investment. In the past, securities class action litigation has often been instituted against companies following periods of volatility in the market price of a company's securities. This type of litigation, if instituted, could result in substantial costs and a diversion of management's attention and resources, which would harm our business, operating results or financial condition.

We do not intend to pay dividends on our common stock so any returns will be limited to the value of our stock.

We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. Any return to stockholders will therefore be limited to the appreciation of their stock.

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

Prior to this offering, our executive officers, directors, and 5% stockholders beneficially owned approximately 79.4% of our voting stock as of September 30, 2014, and, upon the closing of this offering, that same group will hold approximately 44.5% of our outstanding voting stock, assuming (1) conversion of all outstanding shares of convertible preferred stock into 12,224,819 shares of common stock, (2) no exercise of the underwriters' option to purchase additional shares, (3) election by all of our executive officers, directors or 5% stockholders that are holders of Series B convertible preferred stock to have their accrued dividends converted into common stock in connection with this offering, (4) net exercise by all of our executive officers, directors or 5% stockholders that are holders of warrants to purchase common stock of such warrants in full in connection with this offering, and (5) exercise by all of our executive officers, directors or 5% stockholders that are holders of warrants to purchase Series C convertible preferred stock of such warrants in full in connection with this offering, in each case based on the initial public offering price of \$19.00 per share. Therefore, even after this offering, these stockholders will have the ability to influence us through this ownership position. These stockholders may be able to determine all matters requiring

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stockholder approval. For example, these stockholders may be able to control elections of directors, amendments of our organizational documents, or approval of any merger, sale of assets, or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may feel are in your best interest as one of our stockholders.

If you purchase our common stock in this offering, you will incur immediate and substantial dilution in the book value of your shares.

The initial public offering price is substantially higher than the net tangible book value per share of our common stock. Investors purchasing common stock in this offering will pay a price per share that substantially exceeds the book value of our tangible assets after subtracting our liabilities. As a result, investors purchasing common stock in this offering will incur immediate dilution of \$12.07 per share, based on the initial public offering price of \$19.00 per share. Further, investors purchasing common stock in this offering will contribute approximately 50% of the total amount invested by stockholders since our inception, but will own only approximately 28.4% of the shares of common stock outstanding after giving effect to this offering.

This dilution is due to our investors who purchased shares prior to this offering having paid substantially less when they purchased their shares than the price offered to the public in this offering and the exercise of stock options granted to our employees. To the extent outstanding options or warrants are exercised, there will be further dilution to new investors. As a result of the dilution to investors purchasing shares in this offering, investors may receive significantly less than the purchase price paid in this offering, if anything, in the event of our liquidation. For a further description of the dilution that you will experience immediately after this offering, see Dilution.

We are an emerging growth company, and we cannot be certain if the reduced reporting requirements applicable to emerging growth companies will make our common stock less attractive to investors.

We are an emerging growth company, as defined in the Jumpstart Our Business Startups Act, or the JOBS Act, enacted in April 2012. For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, as amended, or the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in this prospectus and our periodic reports and proxy statements and exemptions from the requirements of holding nonbinding advisory votes on executive compensation and stockholder approval of any golden parachute payments not previously approved. We could be an emerging growth company for up to five years following the year in which we complete this offering, although circumstances could cause us to lose that status earlier. We will remain an emerging growth company until the earlier of (1) the last day of the fiscal year (a) following the fifth anniversary of the completion of this offering, (b) in which we have total annual gross revenue of at least \$1 billion or (c) in which we are deemed to be a large accelerated filer, which requires the market value of our common stock that is held by non-affiliates to exceed \$700 million as of the prior June 30th, and (2) the date on which we have issued more than \$1 billion in non-convertible debt during the prior three-year period.

Even after we no longer qualify as an emerging growth company, we may still qualify as a smaller reporting company which would allow us to take advantage of many of the same exemptions from disclosure requirements including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation in this prospectus and our periodic reports and proxy statements. We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

Under the JOBS Act, emerging growth companies can also delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies. As a result, changes in rules of U.S. generally accepted accounting principles or their interpretation, the adoption of new guidance or the application of existing guidance to changes in our business could significantly affect our financial position and results of operations.

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

As a public company, we will incur significant legal, accounting and other expenses that we did not incur as a private company. We will be subject to the reporting requirements of the Securities Exchange Act of 1934, which will require, among other things, that we file with the Securities and Exchange Commission, or the SEC, annual, quarterly and current reports with respect to our business and financial condition. In addition, the Sarbanes-Oxley Act, as well as rules subsequently adopted by the SEC and The NASDAQ Global Market to implement provisions of the Sarbanes-Oxley Act, impose significant requirements on public companies, including requiring establishment and maintenance of effective disclosure and financial controls and changes in corporate governance practices. Further, in July 2010, the Dodd-Frank Wall Street Reform and Consumer Protection Act, or the Dodd-Frank Act, was enacted. There are significant corporate governance and executive compensation related provisions in the Dodd-Frank Act that require the SEC to adopt additional rules and regulations in these areas such as say on pay and proxy access. Recent legislation permits emerging growth companies to implement many of these requirements over a longer period and up to five years from the pricing of this offering. We intend to take advantage of this new legislation but cannot guarantee that we will not be required to implement these requirements sooner than budgeted or planned and thereby incur unexpected expenses. Stockholder activism, the current political environment and the current high level of government intervention and regulatory reform may lead to substantial new regulations and disclosure obligations, which may lead to additional compliance costs and impact the manner in which we operate our business in ways we cannot currently anticipate.

We expect the rules and regulations applicable to public companies to substantially increase our legal and financial compliance costs and to make some activities more time-consuming and costly. If these requirements divert the attention of our management and personnel from other business concerns, they could have a material adverse effect on our business, financial condition and results of operations. The increased costs will decrease our net income or increase our net loss, and may require us to reduce costs in other areas of our business or increase the prices of our products or services. For example, we expect these rules and regulations to make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to incur substantial costs to maintain the same or similar coverage. We cannot predict or estimate the amount or timing of additional costs we may incur to respond to these requirements. The impact of these requirements could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, our board committees or as executive officers.

We have identified a material weakness in our internal control over financial reporting. If our internal control over financial reporting is not effective, we may not be able to accurately report our financial results or file our periodic reports in a timely manner, which may cause investors to lose confidence in our reported financial information and may lead to a decline in our stock price.

Effective internal control over financial reporting is necessary for us to provide reliable financial reports in a timely manner. In connection with the audits of our financial statements for the years ended December 31, 2012 and 2013, we concluded that there was a material weakness in our internal control over financial reporting. A material weakness is a significant deficiency, or a combination of significant deficiencies, in internal control over financial reporting such that it is reasonably possible that a material misstatement of the annual or interim financial statements will not be prevented or detected on a timely basis.

The material weakness related to (1) a lack of internal controls over accounting and financial reporting, particularly surrounding nonroutine transactions and financial reporting, (2) a lack of sufficient staff, including the lack of a chief financial officer or other senior finance executive, and (3) a lack of formalized accounting policy and procedure documentation that is followed by accounting personnel. The material weakness resulted in adjustments that were

primarily related to non-routine transactions and impacted intangible assets, prepaid manufacturing costs, accrued liabilities, equity, expenses and income taxes.

In an attempt to remediate our resource weakness, we have hired a chief financial officer and plan to hire additional finance and accounting personnel to augment our accounting staff and to provide more resources for complex accounting matters and financial reporting. In addition, we are working to establish a standard accounting and operation procedures manual outlining the corporate policies and accounting practices to be followed. However, we cannot assure you that these efforts will remediate our material weakness in a timely manner, or at all, or prevent

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restatements of our financial statements in the future. If we are unable to successfully remediate our material weakness or identify any future significant deficiencies or material weaknesses, the accuracy and timing of our financial reporting may be adversely affected, we may be unable to maintain compliance with securities law requirements regarding timely filing of periodic reports, and our stock price may decline as a result.

In addition, even if we remediate our material weakness, following the completion of this offering, we will be required to expend significant time and resources to further improve our internal controls over financial reporting, including by further expanding our finance and accounting staff. If we fail to adequately staff our accounting and finance function to remediate our material weaknesses and our significant deficiency or otherwise to meet the demands that will be placed upon us as a public company, including the requirements of the Sarbanes-Oxley Act, or fail to maintain adequate internal control over financial reporting, any new or recurring material weakness could prevent our management from concluding our internal control over financial reporting is effective and impair our ability to prevent material misstatements in our financial statements, which could cause our business to suffer.

Changes in accounting rules, assumptions and/or judgments could materially and adversely affect us.

Accounting rules and interpretations for certain aspects of our operations are highly complex and involve significant assumptions and judgment. These complexities could lead to a delay in the preparation and dissemination of our financial statements. Furthermore, changes in accounting rules and interpretations or in our accounting assumptions and/or judgments, such as asset impairments, could significantly impact our financial statements. In some cases, we could be required to apply a new or revised standard retroactively, resulting in restating prior period financial statements. Any of these circumstances could have a material adverse effect on our business, prospects, liquidity, financial condition and results of operations.

Sales of a substantial number of shares of our common stock by our existing stockholders in the public market could cause our stock price to fall.

If our existing stockholders sell, or indicate an intention to sell, substantial amounts of our common stock in the public market after the lock-up and other legal restrictions on resale discussed in this prospectus lapse, the trading price of our common stock could decline. Based on shares of common stock outstanding as of September 30, 2014, upon the closing of this offering we will have outstanding a total of 25,849,571 shares of common stock. Of these shares, only the shares of common stock sold in this offering by us, plus any shares sold upon exercise of the underwriters' option to purchase additional shares, will be freely tradable without restriction in the public market immediately following this offering. Jefferies LLC, and Citigroup Global Markets Inc. however, may, in their sole discretion, permit our officers, directors and other stockholders who are subject to these lock-up agreements to sell shares prior to the expiration of the lock-up agreements.

We expect that the lock-up agreements pertaining to this offering will expire 180 days from the date of this prospectus. After the lock-up agreements expire, up to an additional 14,349,205 shares of common stock will be eligible for sale in the public market, of which 5,698,431 shares are held by directors, executive officers and other affiliates and will be subject to volume limitations under Rule 144 under the Securities Act of 1933, as amended, or the Securities Act. In addition, shares of common stock that are either subject to outstanding options or reserved for future issuance under our employee benefit plans will become eligible for sale in the public market to the extent permitted by the provisions of various vesting schedules, the lock-up agreements and Rule 144 and Rule 701 under the Securities Act. If these additional shares of common stock are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline.

After this offering, the holders of approximately 16.9 million shares of our common stock as of September 30, 2014 will be entitled to rights with respect to the registration of their shares under the Securities Act, subject to the 180-day lock-up agreements described above. See Description of Capital Stock Registration Rights. Registration of these shares under the Securities Act would result in the shares becoming freely tradable without restriction under the Securities Act, except for shares held by affiliates, as defined in Rule 144 under the Securities Act. Any sales of securities by these stockholders could have a material adverse effect on the trading price of our common stock.

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

We expect that significant additional capital may be needed in the future to continue our planned operations, including conducting clinical trials, commercialization efforts, expanded research and development activities and costs associated with operating a public company. To raise capital, we may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time. If we sell common stock, convertible securities or other equity securities, investors may be materially diluted by subsequent sales. Such sales may also result in material dilution to our existing stockholders, and new investors could gain rights, preferences and privileges senior to the holders of our common stock, including shares of common stock sold in this offering.

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

Our management will have broad discretion in the application of the net proceeds from this offering, including for any of the purposes described in the section entitled Use of Proceeds, and you will not have the opportunity as part of your investment decision to assess whether the net proceeds are being used appropriately. Because of the number and variability of factors that will determine our use of the net proceeds from this offering, their ultimate use may vary substantially from their currently intended use. Our management might not apply our net proceeds in ways that ultimately increase the value of your investment. We intend to use the net proceeds from this offering to fund (1) our ongoing and planned Phase 1/2 clinical trials of BPX-501; (2) pre-clinical