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

Title of each class	Name of each exchange on which registered
Common Stock, par value \$0.01 per share	The NASDAQ Global Market

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

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes  No

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. Yes  No

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

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Website, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§229.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes  No

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

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

Large accelerated filer

Accelerated filer

Non-accelerated filer  (Do not check if a smaller reporting company) Smaller reporting company

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

The registrant did not have a public float on the last business day of its most recently completed second fiscal quarter because there was no public market for the registrant's common equity as of such date.

The number of outstanding shares of the registrant's common stock as of March 15, 2015 was 26,378,474.

#### DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant's definitive Proxy Statement for the registrant's 2015 Annual Meeting of Stockholders to be filed with the Securities and Exchange Commission within 120 days after registrant's fiscal year ended December 31, 2014 are incorporated by reference into Part III of this report.



BELLICUM PHARMACEUTICALS, INC.

Form 10-K

For the Fiscal Year Ended December 31, 2014

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## PART I

### Forward-Looking Statements

This Annual Report on Form 10-K, including the sections entitled “Business,” “Risk Factors” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations,” may contain “forward-looking statements.” We may, in some cases, use words such as “anticipate,” “believe,” “could,” “estimate,” “expect,” “intend,” “may,” “plan,” “predict,” “project,” “should,” “will,” “would” or the negative of those terms, and similar expressions that convey uncertainty of future events or outcomes to identify these forward-looking statements. Any statements contained herein that are not statements of historical facts may be deemed to be forward-looking statements. Forward-looking statements in this Annual Report include, but are not limited to, statements about:

- the success, cost and timing of our product development activities and clinical trials;
- our ability to advance CID-based technologies, including CaspaCIDE, CIDE CAR, GoCAR-T and DeCIDE;
- our ability to obtain and maintain regulatory approval of BPX-501 and any other product candidates, and any related restrictions, limitations and/or warnings in the label of an approved product candidate;
- our ability to obtain funding for our operations, including funding necessary to complete further development and commercialization of our product candidates;
- the commercialization of our product candidates, if approved;
- our plans to research, develop and commercialize our product candidates;
- our ability to attract collaborators with development, regulatory and commercialization expertise;
- future agreements with third parties in connection with the commercialization of our product candidates and any other approved product;
- the size and growth potential of the markets for our product candidates, and our ability to serve those markets;
- the rate and degree of market acceptance of our product candidates;
- regulatory developments in the United States and foreign countries;
- our ability to contract with third-party suppliers and manufacturers and their ability to perform adequately;
- the success of competing therapies that are or may become available;
- our ability to attract and retain key scientific or management personnel;
- our ability to grow our organization and increase the size of our facilities to meet our anticipated growth;
- the accuracy of our estimates regarding expenses, future revenue, capital requirements and needs for additional financing;
- our expectations regarding the period during which we qualify as an emerging growth company under the Jumpstart Our Business Startups Act of 2012, or the JOBS Act;
- our use of the proceeds from our recently completed initial public offering; and
- our expectations regarding our ability to obtain and maintain intellectual property protection for our product candidates.

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

You should carefully read this Annual Report and the documents that we reference in this Annual Report completely and with the understanding that our actual future results may be materially different from what we expect. We qualify

all of the forward-looking statements in this Annual Report by these cautionary statements.

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Except as required by law, we undertake no obligation to update these forward-looking statements publicly, or to update the reasons that actual results could differ materially from those anticipated in any forward-looking statements, whether as a result of new information, future events or otherwise.

## ITEM 1. Business

### 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.

We are developing next-generation product candidates in some of the most important areas of cellular immunotherapy, including hematopoietic stem cell transplantation, or 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 chimeric antigen receptors, or 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, 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, play in the immune system, they are difficult to activate appropriately and as a result their use has delivered only modest therapeutic benefit.

Our proprietary CID platform 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:

- CaspaCIDE is our safety switch, incorporated into our HSCT and T-cell receptor, or 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.

CIDeCAR consists of CAR T cells modified to include our CaspaCIDE safety switch and in which the CAR T cell 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 most investigational CAR T cell therapies. 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.

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 extending the interval between rimiducid doses and/or reducing the dosage per infusion.

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·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.

·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. BPX-501 is currently being evaluated in multiple Phase 1/2 clinical trials in the United States and Europe to assess whether BPX-501 T cells from haplo-identical donors administered following HCST are safe and can help speed immune reconstitution. The initial top-line data from ongoing studies is expected by the end of 2015.

·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 metastatic castrate-resistant prostate cancer or, 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:

·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.

·BPX-601. We are developing a GoCAR-T product candidate, BPX-601, for solid tumors overexpressing the prostate stem cell antigen, or 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.

·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 sarcomas and neuroblastomas. 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 by the end 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.

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## Recent Developments

On December 23, 2014, the closing of our initial public offering triggered an acceleration of the payment of \$15 million due to ARIAD Pharmaceuticals, Inc., or ARIAD, under our omnibus amendment agreement with ARIAD and our promissory note issued to ARIAD. As a result of such acceleration, on December 29, 2014, we paid to ARIAD an aggregate amount of \$35 million, which included an additional payment of \$20 million to extinguish the promissory note. In connection with the note payments and pursuant to the omnibus amendment agreement, ARIAD returned to us all of the 677,463 shares of our common stock then held by ARIAD.

## Cellular Immunotherapy

Cellular immunotherapy harnesses a patient's own immune cells to attack and eliminate harmful disease cells in the body. The immune system is the body's defense network. It consists of a number of cells and organs that, working together, recognize and respond to threats in the form of pathogens. T cells are a type of white blood cell that recognize pathogens and can target and eliminate them upon full activation through the addition of appropriate co-stimulatory signals.

Dendritic cells, another component of the immune system, are antigen-presenting cells found in skin and other tissues like the lining of the gut that can sense and respond to the environment. Dendritic cells engulf and process potential threats they encounter, presenting them as antigens to T cells and B cells to allow the body to mount an immune response.

The following three therapeutic applications of cellular immunotherapy have been the primary areas of research and development by research institutes and biopharmaceutical companies, given their promise of effectively treating patients suffering from severe and life-threatening diseases.

**HSCT.** HSCT is the transplantation of stem cells and other immune cells derived from bone marrow, peripheral blood or umbilical cord blood. The transplantation may be autologous, using the patient's own cells, or allogeneic, using a donor's cells. HSCT is often the only curative option for a wide range of treatment-refractory hematological cancers, such as ALL, acute myeloid leukemia, or AML, and chronic myeloid leukemia, or CML. HSCT is also used as a high-risk treatment for orphan inherited blood disorders, such as sickle cell disease, beta-thalassemia and certain immune disorders.

**Dendritic Cell Therapy.** Whereas HSCT and CAR T cells involve direct administration of T cells to the patient, dendritic cell therapies are designed to indirectly stimulate T cells already present in the patient. Given the important role of dendritic cells in initiating an immune response in the body, substantial research has been conducted to leverage the attributes of dendritic cells to treat cancer. Cancer vaccines are the most common form of dendritic cell-based therapy. These vaccines entail collecting certain monocytes, a type of white blood cell, from the patient's body, maturing them into dendritic cells, "loading" them ex vivo with the patient's cancer antigens, and sometimes modifying them in other ways to improve their potency, and then re-infusing the modified dendritic cells in the patient.

**Genetically Modified T-cell Therapy (CAR-T and TCR).** This approach entails collecting a patient's T cells, genetically modifying them ex vivo, or outside of the body, to incorporate specific receptors which target cancer cells and then re-infusing the modified T cells back into the patient. Two types of cancer-specific receptors are typically used, CARs that recognize whole antigens on the surface of cancer cells, and TCRs that bind to cancer-associated peptides, or fragments of proteins, from either inside or on the surface of the cancer cells. In early human clinical trials, CAR T cell therapy has demonstrated an unprecedented ability to achieve durable complete responses in some leukemias and lymphomas, even in patients who have suffered multiple relapses.



## Limitations of Current Cellular Immunotherapy Approaches.

Despite rapid advances in various approaches to cellular immunotherapy and the biopharmaceutical industry's considerable investment in research and development, certain challenges have prevented these therapies from realizing their maximum potential. Some of these obstacles and issues are highlighted below:

### Our Proprietary CID Technology Platform

Our proprietary CID technology platform is designed to address the challenges of current cellular immunotherapies. Cellular activities and functions, such as growth, activation, proliferation and cell death, are controlled by cascades of specialized signaling proteins. Our CID platform consists of molecular switches, modified forms of these signaling proteins, which are triggered inside the patient by infusion of a small molecule, rimiducid, instead of by natural upstream signals. Our product candidates are based on either a "safety switch," or an "activation switch." After rimiducid is administered, the "safety switch" is designed to lead to programmed cell death, or apoptosis, and the "activation switch" is designed to lead to proliferation and/or activation of immune cells.

We incorporate the molecular switches in the appropriate immune cells and administer them to the patient. After the modified immune cells are inside the patient's body, specific functions of these cells may be controlled by administering rimiducid by intravenous, or IV, infusion. Rimiducid has been designed to bind to a specifically designed domain of CID switch proteins. Once introduced, rimiducid couples, or dimerizes, CID switch proteins together to create a cluster that triggers the signaling cascade. Aside from its impact on CID-modified immune cells bearing switch proteins, rimiducid has no other known effect on the body. To date, rimiducid has been used in more than 150 infusions in humans without any reported serious adverse events related to rimiducid.

Our proprietary CID-based product candidates depend on the following signaling molecules to trigger signaling cascades, resulting in different cell activities:

- Caspase-9: Signaling Molecule for Apoptosis. Caspase-9 is the initiating enzyme in the apoptosis pathway. When activated, caspase-9 starts a signaling cascade, including the activation of caspase-3, which ultimately leads to apoptosis, a non-inflammatory process of cell elimination.
- MyD88/CD40: Signaling Molecules for Activation and Proliferation. Myeloid differentiation primary response gene, or MyD88, is a protein that has functions in cellular responses to stimuli such as stress, cytokines and bacteria or viruses. CD40 is a co-stimulatory protein found on antigen-presenting cells, such as dendritic cells and B cells and is required for their activation. Although the effects of MyD88 and CD40 have been studied previously in dendritic cell therapies, our novel approach applies them to T cell based immunotherapies.

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## Our Proprietary Switch Technologies

With the CID platform as the foundation, we have created different molecular switch technologies customized for specific cellular immunotherapy approaches and therapeutic indications. The table below summarizes our key switch technologies.

### CaspaCIDE

CaspaCIDE is our CID safety switch technology designed to eliminate cells in the event of toxicity. The CaspaCIDE switch consists of the CID-binding domain coupled to the signaling domain of caspase-9, an enzyme that is part of the apoptotic pathway. Infusion of rimiducid is designed to trigger activation of this domain of caspase-9 (iCasp9), which in turn leads to selective apoptosis of the CaspaCIDE-containing cells. Because CaspaCIDE is designed to be permanently incorporated into our cellular therapies, the safety switch has the potential to be available for use long after the initial therapy is delivered. This technology is applied to our lead clinical product candidate, BPX-501, an adjunct T-cell therapy after allogeneic HSCT, and to our TCR product candidate, BPX-701.

We believe that CaspaCIDE is the optimal cell therapy safety switch technology. The only other widely reported approach used in the clinic is based on the Herpes simplex virus thymidine kinase, or HSV-tk, a non-human and as such immunogenic protein which is activated to kill the cell by the widely-used anti-viral drug, ganciclovir. Comparative studies have demonstrated CaspaCIDE's superiority to HSV-tk, based on lack of immunogenicity, effectiveness in rescuing animals from toxicities that have progressed, lack of dependence on the cell cycle for cell elimination, and most importantly, speed of elimination. In human trials, CaspaCIDE has demonstrated clinical efficacy in human patients beginning as soon as 30 minutes after administration of the activating drug, rimiducid. Lastly, rimiducid is bio-inert in the absence of cells containing a CID switch, and has no other clinical use; ganciclovir has side effects, and physicians are reluctant to lose the ability to use it to treat viral infections in patients treated with cells containing HSV-tk.

Other cell elimination approaches described in the literature include gene modification of cells to express truncated epidermal growth factor receptor, or EGFRt, or codon-optimized CD20. Administration of the monoclonal antibodies cetuximab or rituximab, respectively, is intended to trigger antibody-dependent cellular cytotoxicity, or ADCC, mediated cell elimination. While CaspaCIDE eliminates cells via the apoptotic pathway, the body's non-inflammatory mechanism for this important function, we believe an ADCC-mediated mechanism may add to complications in patients already in an inflammatory crisis, such as seen with serious cytokine

release syndrome after CAR T cell therapy. Moreover, cetuximab and rituximab, both anti-cancer therapies that have potentially serious side effects, are unlikely to be usable in a titratable manner. Lastly, these approaches have yet to demonstrate efficacy in clinical trials.

CaspaCIDE has been evaluated in both preclinical and clinical studies, with additional Phase 1/2 clinical trials ongoing and planned. In addition to using our CaspaCIDE technology for the substantial elimination of cellular therapy (like an “off” switch), we are studying partial elimination of a cellular therapy (like a “dimmer” switch) by delivering reduced doses of rimiducid. We observed the dose response to rimiducid by measuring the viability of BPX-501 cells in culture following the addition of increasing amounts of rimiducid to the culture medium as well as by measuring the survival of BPX-501 cells in vivo in immune-deficient mice following injection of increasing doses of rimiducid. In these preclinical studies, rimiducid rapidly and consistently reduced or eliminated CaspaCIDE-containing cells in a dose-dependent manner.

In addition to our internal preclinical and clinical development activities, we are collaborating with renowned cancer research centers with expertise in cellular immunotherapy to apply our CaspaCIDE safety switch to the collaborators’ CAR-T product candidates. The National Cancer Institute, or NCI, has initiated a Phase 1/2 clinical trial for sarcoma and other solid tumors with a CAR construct targeting a solid tumor antigen combined with CaspaCIDE. Although we are not the sponsor of this clinical trial, we believe that it may extend clinical proof of principle for CaspaCIDE from the HSCT setting to the CAR T cell setting.

#### CIDeCAR

CIDeCAR consists of a CAR T cell that utilizes MC, our proprietary novel dual co-stimulatory domain, for improved T-cell activation and proliferation, and the CaspaCIDE safety switch. CAR interaction with cancer cell antigens complements MC signaling, which then leads to activation of T cells. In the event of serious toxicity, rimiducid activation of caspase-9 is designed to eliminate the CIDeCAR T cells.

In order to improve the effectiveness of CAR T cells in settings other than blood cancers located principally in the bone marrow, such as leukemia, some researchers have been working to develop “armored CARs” in which supplemental co-stimulatory signals or pro-inflammatory cytokines are added to the CAR T cells. Like an “armored CAR,” we include MC in our CIDeCAR technology in order to increase the potency of the therapy in these indications. While promising, these approaches may exacerbate safety issues found in standard CARs, such as cytokine release syndrome. We incorporate CaspaCIDE into CIDeCAR to address these safety concerns.

In proof-of-principle preclinical studies of CIDeCAR technology, CIDeCAR candidate BPX-401 and a solid tumor CIDeCAR targeting Her2, both of which incorporate MC, in place of the standard co-stimulatory molecules CD28, 4-1BB, or both together, were evaluated in vitro. These preclinical studies show that CIDeCAR technology results in enhanced activation, proliferation and tumor cell killing compared to standard comparator CARs. In addition, these studies demonstrate elimination of the CIDeCAR T cells after exposure to rimiducid.

Preclinical animal studies have shown that BPX-401 cells exhibit both anti-tumor activity and partial or complete elimination of T cells after administration of rimiducid in an NSG mouse Raji tumor model.

#### GoCAR-T

Our GoCAR-T technology incorporates a switch that activates CAR T cells when triggered by both rimiducid and the targeted antigen expressed on the surface of the cancer cells. Current generation CAR T cell constructs consist of a CD3 domain and one or more co-stimulatory molecules that are both activated when a cancer antigen binds to the portion of the chimeric antigen receptor on the outside of the engineered T cell. This reliance on antigen for activation of the CAR T cell results in an unpredictable and inherently uncontrollable therapeutic effect. For example, CAR T cells that target the CD19 receptor have been shown to proliferate in excess of 100,000-fold in some patients, to

comprise over 50% of circulating lymphocytes. Solid tumor CAR T cells, on the other hand, often fail to proliferate or persist at all for more than a few days or weeks and have been largely ineffective. In each situation, the physician has no effective way to intervene to achieve greater consistency, once the cells have been administered.

Our GoCAR-T technology is designed to change the current paradigm by separating the CIDECAR dual co-stimulatory domain, MC, from the antigen recognition domain and moving it onto a separate molecular switch that can be controlled by rimiducid. GoCAR-T cells are designed to only be fully activated when exposed to both the cancer cells and rimiducid. This separation is designed to control the degree of activation of the CAR T cells through adjustments to the schedule of rimiducid administration, but still in a tumor-dependent manner.



In a proof-of-principle in vitro study of our GoCAR-T technology, GoCAR-T cells targeting the PSCA antigen can only be fully activated when the GoCAR-T cells are exposed to both their target PSCA-expressing human pancreatic cancer cells and rimiducid. In in vivo studies of GoCAR-T technology, target antigen PSCA-expressing Capan-1 human pancreatic tumors were established in immune-deficient, or NSG, mice were controlled by administration of GoCAR-T cells targeting PSCA and twice weekly administration of rimiducid.

We believe these studies together provide proof-of-principle that GoCAR-T technology may allow rimiducid to modulate the therapeutic effect from initiation of treatment, turning CAR T cell therapy from an uncontrollable, and largely unpredictable class into a more predictable therapy which can be adjusted, like a small molecule, to the patient's therapeutic window to the appropriate level.

## DeCIDE

DeCIDE technology is used to control the activation of dendritic cells. Dendritic cells are an important part of the immune system, processing antigens for presentation to T cells. Optimal stimulation of dendritic cells requires the activation of both the CD40 and toll-like receptor, or TLR, pathways, which results in maturation and activation of the dendritic cells as well as production of key cytokines, such as IL-12. These processes lead to a therapeutic response to the antigen by the patient's immune system. The potency of an immune response is governed by the maturation of dendritic cells in the patient's lymph nodes as well as the duration of interaction between activated dendritic cells with circulating T cells.

To take control of the activation of the dendritic cells and the resulting immune response to cancer, we have taken the signaling domains of CD40 and MyD88, and coupled them to our CID binding domain, to create our inducible MC switch, which we then insert into dendritic cells along with the PSMA antigen. Upon exposure to rimiducid, DeCIDE-containing dendritic cells are designed to become highly activated in a process that is no longer susceptible to being turned off by MMP. Our DeCIDE technology, thus, potentially enables us to activate dendritic cells with rimiducid after the patient has been vaccinated and the dendritic cells have migrated to the draining lymph nodes in a potent and long-lasting manner.

Fully activated dendritic cells exhibit a number of important traits, including increases to the levels of important cell surface markers, and production of important cytokines, such as IL-12. Cultured BPX-201 cells, which are dendritic cells transduced with our DeCIDE switch technology, produce increased levels of IL-12 in response to rimiducid. These data suggest that in addition to the temporal control of dendritic cell activation that DeCIDE technology affords, once exposed to rimiducid, DeCIDE-containing dendritic cells become highly activated, which may lead to more potent anti-cancer activity in patients.

## Our Product Candidates

### BPX-501: CaspaCIDE Product Candidate for Hematological Diseases

BPX-501 is an adjunct T-cell therapy administered after allogeneic HSCT that incorporates our CaspaCIDE technology. We are developing BPX-501 in the initial indications of hematological cancers and orphan inherited blood disorders. In the indication of hematological cancers, we are pursuing two regulatory pathways: (1) support of immune system recovery following allogeneic HSCT, and (2) the treatment of the relapse of underlying disease following allogeneic HSCT. In orphan inherited blood disorders, we are pursuing a parallel regulatory pathway for immune system recovery following allogeneic HSCT.

We are currently conducting three Phase 1/2 clinical trials of BPX-501 at leading transplant centers in the United States and Europe: BP-001, a clinical trial in adults in which BPX-501 is administered after initial allogeneic HSCT for hematological cancers, BP-003, a clinical trial in children with orphan inherited blood disorders in which BPX-501 is administered after initial allogeneic HSCT, and BP-004 an additional Phase 1/2 clinical trial in children with

hematological cancers or orphan inherited blood disorders. In addition, we are planning to initiate additional Phase 1/2 clinical trials in the United States and Europe in 2015, as part of our strategy to pursue a global regulatory approval and expand the potential addressable patient population for BPX-501.

#### BPX-201: DeCIDE Cancer Vaccine Product Candidate

We are developing BPX-201 as a dendritic cell cancer vaccine designed to treat mCRPC. BPX-201 is an autologous therapy, in which the patient's own white blood cells are extracted and modified *ex vivo*. The cells are matured and then genetically engineered to express the DeCIDE switch domains and the PSMA antigen. Then, the modified cells are washed, apportioned into individual doses, and frozen for later administration to the patient.

By incorporating the DeCIDE switch that activates therapy only in the presence of rimiducid, physicians may be able to strategically time the immune system's attack on cancer cells. The rationale behind this approach is to allow BPX-201 cells to bypass critical immune checkpoints that can potentially reduce therapeutic effect and migrate to nearby lymph nodes to initiate a potent and durable antigen-specific T-cell response.

We submitted an IND for BPX-201 in September 2012 and it is currently being evaluated in an 18-patient Phase 1 clinical trial for mCRPC. The clinical trial design consists of three cohorts of six patients each, who will receive escalating doses of BPX-201. The patients will be followed for two years after enrollment.

We are evaluating opportunities for BPX-201 in combination with other cancer immunotherapies, such as checkpoint inhibitors. Checkpoint inhibitors act by removing inhibitory signals on antigen- or tumor- specific T cells already present in the patient's body. BPX-201 shows the potential to stimulate proliferation of PSMA-specific T cells thereby providing a rationale to successfully combine BPX-201 with checkpoint inhibitors.

#### BPX-401: CIDE CAR Product Candidate for Hematological Cancers

We are developing BPX-401 for the treatment of hematological cancers expressing the CD19 antigen, such as ALL, CLL and certain types of non-Hodgkin's lymphoma. We have generated preclinical proof-of-principle data in vitro showing that BPX-401 has significant CAR T cell activation and proliferation potential, and may be more effective in killing cancer cells compared to other CAR-T constructs. We intend to file an IND and initiate a Phase 1/2 clinical trial in the first half of 2016.

The current standard of care in these indications, chemotherapy combined with monoclonal antibody therapies, works to varying degrees with high disease relapse rates. CD19-targeted CAR-T therapies have elicited high objective response rates in some of these B cell cancers, but they have demonstrated major safety risks.

#### BPX-601: GoCAR-T Product Candidate for Solid Tumors

We are currently conducting preclinical studies of BPX-601 for the treatment of solid tumors overexpressing the PSCA antigen. PSCA is highly expressed in some pancreatic cancers, as well as in a portion other solid tumors, including bladder, esophageal and gastric cancers. Although many product candidates are in development for these cancers, there are currently no approved products targeting PSCA. We intend to initiate a Phase 1/2 clinical trial in the second half of 2016. In order to commercialize this product candidate, we may need to obtain an additional intellectual property license.

#### BPX-701: CaspaCIDE TCR Product Candidate for Solid Tumors

We are developing BPX-701, a TCR-based therapy that incorporates our CaspaCIDE technology, in collaboration with Leiden University Medical Center, for the treatment of PRAME-expressing melanoma, sarcomas and neuroblastomas. We intend to file an IND and initiate a Phase 1/2 clinical trial by the end of 2015. Clinical sites for this trial have been identified.

BPX-701 is designed to target preferentially-expressed antigen in melanoma, or PRAME, a gene that is predominantly expressed in human melanomas but not in normal tissues. As initially reported in Clinical Cancer Research 2011, PRAME-specific clones showed high reactivity against a panel of PRAME positive tumor cell lines, metastatic melanoma, sarcomas and neuroblastoma tissues, and no reactivity against normal cell types, with the exception of low reactivity against kidney epithelial cells and intermediate reactivity against mature dendritic cells, or DCs. In other TCR programs, despite careful evaluation of normal tissues to identify potential off target effects, unexpected cross-reactivities have been encountered in clinical trials, leading to serious adverse events including patient deaths. BPX-701 containing the CaspaCIDE safety switch, has demonstrated complete elimination in response to rimiducid. Therefore, we believe a PRAME-TCR with CaspaCIDE can provide safety in the clinical development of this TCR.

#### Other Development Programs

We believe that our CIDE CAR, GoCAR-T and CaspaCIDE TCR technologies have broad applicability against a range of cancer targets which form the basis for additional development programs, some of which are described below:

#### CIDeCAR for Solid Tumors

Beyond hematological cancers, we are studying the full potential of CIDeCAR to enable treatment of more challenging solid tumor cancers in which concerns regarding toxicity are paramount in the field of cell therapy. To this end, we are conducting preclinical studies of various CIDeCAR product candidates targeting solid tumor antigens.

#### CaspaCIDE TCR for Hematological Cancers

We are working with our collaborator, Leiden University Medical Center, to evaluate an additional TCR with high affinity for certain peptides for the treatment of hematological cancers, including ALL, CLL, MCL and MM. The TCR construct incorporates the CaspaCIDE safety switch.

## Manufacturing, Processing and Delivering to Patients

Our product candidates require a combination of three critical components: (1) viral vectors with DNA content encoded for our proprietary switch proteins and co-stimulatory and other accessory molecules, (2) patient-specific donor T cells or dendritic cells that are genetically modified by our viral vectors, and (3) the synthetic small molecule rimiducid which activates the switch proteins. Each of these components requires a separate supply chain and shares the same regulatory requirements applicable for biological or chemical materials suitable for human use. Details on each of these components are described below:

- **Viral Vectors.** We use a retrovirus to transduce our T cell based product candidates. We believe that the retrovirus is optimal for T cell transduction given that it is an integrating vector that induces long-term gene expression, exhibits high transduction efficiency, has large capacity for DNA content, and has been safely used in clinical trials. As an alternative approach, we are investigating in parallel the use of lentivirus for several of our product candidates. In certain embodiments, lentiviral vectors may provide advantages over retroviral vectors. To transduce dendritic cells, we use a specific type of adenovirus, which has been shown to be efficient at transducing this cell type and is cost-effective to manufacture and scale. The vector production is performed at multiple third-party supplier facilities under GMP procedures and requirements. These suppliers have significant experience and expertise in vector manufacturing and have dedicated capacity to satisfy demand for large clinical trials and product commercialization.
- **Genetically Modified T Cells and Dendritic Cells.** We have agreements with reputable contract manufacturing organizations, or CMOs, with facilities in both the United States and Europe for processing and manufacturing our genetically modified T cells and dendritic cells. We have designed and refined a proprietary process for cell engineering that has been improved from lab-based open procedures used in academic and research settings to a functionally closed system that is more appropriate for large-scale clinical trials and commercialization. Our system is compliant with current guidelines and regulations for cell-based manufacturing in the United States and Europe and has been successfully transferred and implemented by our CMOs.
- **Rimiducid.** Rimiducid is a synthetic small molecule which has been rationally designed to trigger the proprietary switch proteins in our CID platform. We have separate third-party manufacturers for the active pharmaceutical ingredient, or API and the finished drug product. Manufacturers of both the API and finished drug product are licensed to manufacture a variety of marketed drugs worldwide and have been selected based on their ability to provide supplies for our clinical trials and future commercialization.

Given that our product candidates are for patients whose conditions can rapidly deteriorate, we are focused on continuously refining our overall cell therapy process (manufacturing, processing and delivery to patient) to be more efficient.

Our current process cycle from our product candidates, from collection of white blood cells to infusion of the final product, can be completed in as little as two weeks and are customized to be complementary to the treatment procedure of interest in order to prevent any delays or complications.

## Intellectual Property

We seek to protect proprietary technology, inventions, and improvements that are commercially important to our business by seeking, maintaining, and defending patent rights, whether developed internally or licensed from third parties. We also seek to rely on regulatory protection afforded through orphan drug designations, data exclusivity, market exclusivity and patent term extensions where available as well as contractual agreements with our academic and commercial partners.

To achieve this objective, a strategic focus for us has been to identify and license key patents and patent applications that serve to enhance our intellectual property and technology position. Our intellectual property estate includes: (1) claims directed to core CID technologies and components used in our products; (2) claims directed to methods of treatment for therapeutic indications; (3) claims directed to specific products; and (4) claims directed to innovative methods for generating new constructs for genetically engineering T cells and dendritic cells. We believe our patent

estate, together with our efforts to develop and patent next generation technologies, provides us with a substantial intellectual property position.

However, the area of patent and other intellectual property rights in biotechnology is an evolving one with many risks and uncertainties. We are aware there are third party patents having claims that may be considered relevant to the BPX-201 technology for which we are seeking, regulatory approval, however, we believe these patents have a patent term that may expire prior to the time we expect to obtain regulatory approval for this technology. We believe that if claims in one or more of the patents referenced in the previous sentence 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. Please refer to the section entitled “Item 1A. Risk Factors—Risks Related to Our Intellectual Property” herein for associated risks.

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. We believe that our BPX-401 and BPX-601 technologies are not covered by claims of this patent. Please refer to the section entitled “Item 1A. Risk Factors—Risks Related to Our Intellectual Property” herein for associated risks.

We are aware of third party patents having claims that may be considered as being directed to single-chain antibody fragments that bind to PSCA and these patents may be considered relevant to BPX-601 technologies we are developing. We currently are evaluating whether or not we need to obtain rights to these patents under a license, and if it is determined that we need to obtain such rights, whether these rights can be obtained. Please refer to the section entitled “Item 1A. Risk Factors—Risks Related to Our Intellectual Property” herein for associated risks.

To our knowledge, our patent estate, on a worldwide basis, includes 75 issued patents 18 of which are in the United States) and 47 pending patent applications (20 of which are in the United States) which we own or for which we have an exclusive (either in its entirety or within our field of use) commercial license as of March 13, 2015. Of these:

§ We have internally developed technology disclosed in one pending provisional patent application and three utility patent applications in the United States, and three pending international (PCT) patent applications which relate to our CIDECAR technology. If the provisional patent application is converted to a utility patent application, and a U.S. patent issues therefrom, the estimated expiration date of the last to expire patent is in 2035 or later. If patents are issued in foreign jurisdictions, the anticipated expiration date of the last to expire patent will also be in 2035.

§ We have internally developed technology disclosed in two pending utility patent applications in the United States and two pending international (PCT) patent applications which relates to our GoCAR-T technology. If U.S. patents issue from the US applications, the estimated expiration date of the last to expire patent is in 2034 or later. If patents are issued in foreign jurisdictions, the anticipated expiration dates will be in 2034 or later.

§ We have internally developed technology disclosed in two U.S. provisional patent applications, which relates to a “non-inducible” CAR and “non-inducible” co-stimulatory polypeptide, which may also be used in combination with our CIDECAR technology. If either of these provisional patent applications is converted to a utility patent application, and a U.S. patent issues from it, the estimated expiration date of the patent is 2035 or later. If patents are filed and issued in foreign jurisdictions, the anticipated expiration dates will be in 2035 or later.

§ Pursuant to our licenses from Baylor, we have exclusive commercial rights to three issued U.S. patents expiring in 2024 or later, seven pending U.S. utility patent applications, one issued patent in Australia expiring in 2027, one issued patent in Australia expiring in 2031 and 21 pending patent applications in foreign jurisdictions that relate to our GoCAR-T, BPX-201 and certain of our other technologies. If U.S. patents issue from the currently pending U.S. patent applications, the estimated expiration date of the last to expire patent is 2031 or later. Patent applications have been filed in foreign jurisdictions, including Australia, Canada, Europe, Hong Kong and Japan. If patents from the currently pending patent applications are issued in foreign jurisdictions, the estimated expiration dates range from 2024 to 2029.

§ Pursuant to our license agreement with ARIAD, as amended, we have exclusive commercial rights within our field of use to 69 patents (14 in the United States and 55 in foreign jurisdictions, including Australia, Canada, China, Europe, Japan and Korea), which relate to dimerizer technology. The estimated expiration date of the last to expire U.S. patent is February 2016. The estimated expiration dates of the last to expire foreign patents are between 2015<sup>1</sup> and 2020. Also pursuant to this license agreement, we have exclusive commercial rights within our field of use to two pending applications (one in the United States and one in Australia) which relate to dimerizer technology. If a U.S. patent issues from the currently pending U.S. patent application, the estimated expiration date of the last to expire patent is 2032 or later. If a patent issues in Australia from the currently pending application, the estimated expiration date is 2031.

These provisional, pending, or issued patents include composition of matter and/or method of use claims.

As noted above, patent coverage on rimiducid, the dimerization molecule AP1903, will expire in the U.S. in 2016. However, we believe that additional barriers to entry exist for a competitor attempting to use rimiducid after patent expiration. This is significant because, if true, then potential competitors will not be able to use the abbreviated new drug application pathway for approval of rimiducid. With respect to our investigational products, the FDA has assigned combination product status to BPX-501, and we plan to submit a biological license application, or BLA, for the combination product. We believe that this will be the case for each future product candidate of ours that incorporates rimiducid. If our investigational products incorporating rimiducid receive FDA approval through BLAs, then the FDA would not approve any biosimilar of these combination products until at least 12 years from the date that we receive FDA approval. Additionally, although 'biosimilar' provisions exist for products approved through BLAs, it is not clear if the FDA will permit the biosimilar route to be used for complex biological products such as our investigational products.

Rimiducid is a relatively complex drug substance to manufacture. We have substantial experience in manufacturing of rimiducid and in preparing it for patient infusion. Our manufacturing know-how is a valuable asset and we incorporate contractual confidentiality terms in all agreements with our third party manufacturers. We believe that a competitor will face substantial obstacles with respect to time and cost in order to derive a clinically acceptable manufacturing process.

Our strategy is also to develop and obtain additional intellectual property covering manufacturing processes and methods for genetically engineering T cells expressing new constructs. To support this effort, we have established expertise and development capabilities focused in the areas of preclinical research and development, manufacturing and manufacturing process scale-up, quality control, quality assurance, product delivery and storage, regulatory affairs and clinical trial design and implementation. As appropriate, we expect to file additional patent applications to expand this layer of our intellectual property estate.

The term of individual patents depends upon the legal term of the patents in the countries in which they are obtained. In most countries in which we file, the patent term is 20 years from the date of filing of the first non-provisional application to which priority is claimed. In the United States, a patent's term may be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the U.S. Patent and Trademark Office in granting a patent, or may be shortened if a patent is terminally disclaimed over an earlier-filed patent. The term of a patent that covers an FDA-approved drug or biologic may also be eligible for a patent term restoration of up to five years under the Hatch-Waxman Act, which is designed to compensate for the patent term lost during the FDA regulatory review process. The length of the patent term restoration is calculated based on the length of time the drug or biologic is under regulatory review. A patent term restoration under the Hatch-Waxman Act cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval and only one patent applicable to an approved drug or biologic may be restored. Moreover, a patent can only be restored once, and thus, if a single patent is applicable to multiple products, it can only be extended based on one product. Similar provisions are available in Europe and certain other foreign jurisdictions to extend the term of a patent that covers an approved drug or biologic. When possible, depending upon the length of clinical trials and other factors involved in the filing of a BLA we expect to apply for patent term extensions for patents covering our product candidates and their methods of use.

We may rely, in some circumstances, on trade secrets to protect our technology. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors and contractors. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors. To the extent that our



consultants, contractors or collaborators use intellectual property owned by others in their work for us, disputes may arise as to the rights in related or resulting know-how and inventions.

#### Our License Agreements

##### License Agreement with ARIAD Pharmaceuticals, Inc.

##### 2011 License Agreement

On March 7, 2011, we entered into an amended and restated exclusive license agreement, or restated ARIAD license, with ARIAD which restated a license agreement entered into in 2006. Under the restated ARIAD license, ARIAD granted to us an exclusive (even as to the ARIAD) license, with the right to grant sublicenses, under ARIAD's patent rights relating to dimerizers, genetic constructs coding for dimerizer binding domains, vectors containing said constructs, cells containing said constructs and methods of inducing biological processes in cells containing said constructs. These licensed patent rights were limited in the 2011 restated license to defined products in the fields of cell transplantation and certain types of cancer.

In connection with the initial license, in 2006, we issued 121,242 shares of our common stock to ARIAD which were subject to antidilution protection that ultimately resulted in additional issuances to ARIAD by us of 556,221 shares of our common stock, such that ARIAD received a total of 677,463 shares of our common stock under the license agreement. In addition, we paid ARIAD a license fee of \$250,000 in connection with the restatement in 2011. The restated ARIAD license also provided for certain royalty and milestone payments, which were subsequently terminated pursuant to an omnibus amendment agreement with ARIAD (see below).

Under the restated ARIAD license, we are required to diligently proceed with the development, manufacture and sale of licensed products. The restated ARIAD license is subject at all times to restrictions and obligations under a license agreement by and between ARIAD Gene Therapeutics, Inc. (one of ARIAD's affiliates which merged into ARIAD) and the academic institution from which ARIAD obtained its license to the underlying technology. While we are not required to pay royalties or fees to such academic institution, no sub licensee of ours may enter into a sublicense with respect to any intellectual property owned by the academic institution without its consent, which terms must be consistent with those included in the agreement between ARIAD and such academic institution.

The restated ARIAD license will expire upon expiration of the last license term of a licensed product covered by the agreement, which is the later of (1) 12 years from the date of the first commercial sale of the licensed product, or (2) the expiration of the last to expire valid patent claim on the licensed product. Either party to the license may terminate or modify the restated ARIAD license upon a material breach by the other party 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 bankruptcy of the other party. We may terminate the restated ARIAD license in our sole discretion at any time if we determine not to develop or commercialize any licensed product. In addition, upon termination of the restated ARIAD license prior to expiration, we must transfer any ownership and any beneficial ownership in any orphan drug designation or any similar designation in any jurisdiction of orphan drug status of the ARIAD dimerizer to ARIAD.

#### 2014 Amendment

In October 2014, we entered into an omnibus amendment agreement with ARIAD, which in part amended the restated ARIAD license to expand the license to cover a broader scope of dimerizers and licensed products for use and exploitation in any human therapeutic field of use other than in vivo administration of genetic material directly into a human being using viral vectors for the purpose of producing proteins or other macromolecules that are expressed or secreted for therapeutic or prophylactic purposes.

In connection with the amendment, we made an initial payment of \$15,000,000 and we issued a promissory note to ARIAD for a principal amount of \$35,000,000 in return for the broader scope of the license and the termination of all obligations to make milestone and royalty payments to ARIAD in the future. On December 23, 2014, the closing of our initial public offering triggered an acceleration of the payment of \$15,000,000 due to ARIAD under the amendment and the promissory note. As a result of such acceleration, on December 29, 2014, we paid to ARIAD an aggregate amount of \$35,000,000, which included an additional payment of \$20,000,000 to extinguish the promissory note. In exchange, ARIAD returned to us all of the 677,463 shares of our common stock then held by ARIAD and all of the agreements related to ARIAD's rights as a stockholder of us were terminated.

#### License Agreements with Baylor College of Medicine

##### 2008 Baylor License Agreement

Pursuant to an Exclusive License Agreement with Baylor College of Medicine, or Baylor, dated March 20, 2008, or the 2008 Baylor license agreement, we obtained an exclusive, worldwide and fully paid up license to certain intellectual property, including intellectual property related to methods for activating antigen presenting cells and to genetic constructs coding for membrane bound inducible cytoplasmic CD40.

As consideration for the 2008 Baylor license agreement, we issued to Baylor 23,529 shares of our common stock and assumed responsibility for all legal fees and expenses, filing or maintenance fees, assessments and all other costs and expenses related to prosecuting, obtaining and maintaining patent protection on the patents subject to the 2008 Baylor license agreement.

The 2008 Baylor license agreement is subject to certain restrictions and is nonexclusive with respect to (1) the making or use of the licensed intellectual property for use in non-commercial research, patient care, teaching, and other educational purposes; (2) any non-exclusive license covering the licensed intellectual property that Baylor grants to other academic or research institutions for noncommercial research purposes; (3) any non-exclusive licenses that Baylor is required to grant to the U.S. or foreign state pursuant to an existing or future treaty with the U.S.; and (4) a non-exclusive license granted to ARIAD under the terms of a materials transfer agreement between Baylor and ARIAD.

Baylor may terminate or modify the 2008 Baylor license agreement in the event of a material breach 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. We may terminate the 2008 Baylor license agreement, or any portion thereof, at our sole discretion at any time upon 30 days' written notice to Baylor. Upon termination of the 2008 Baylor license agreement, all rights to the intellectual property immediately revert to Baylor.

#### 2010 Baylor License Agreement

Pursuant to an Exclusive License Agreement with Baylor, dated June 27, 2010, or the 2010 Baylor license agreement, we obtained an exclusive, worldwide license to certain intellectual property, including intellectual property related to methods for treating prostate cancer, methods of administering T cells to a patient, and methods of activating antigen presenting cells with constructs comprising MyD88 and CD40.

Pursuant to the terms of the 2010 Baylor license agreement, we paid Baylor a license execution fee of \$30,000. In addition, we are required to pay a low annual maintenance fee on each anniversary of the agreement date.

The terms of the 2010 Baylor license agreement also require us to make royalty payments of less than one percent, subject to certain annual minimums, on net sales of products covered by the license. In addition, to the extent we enter into a sublicensing agreement relating to a licensed product, we are required to pay Baylor a percentage in the mid-single digits on all non-royalty income received from sublicensing revenue. We are required to make milestone payments, of up to \$735,000 in aggregate, upon successful completion of clinical and regulatory milestones regarding the first two products covered by this license.

The 2010 Baylor license agreement will expire upon expiration of the last patent contained in the licensed patent rights, on a country-by-country basis, upon which we will have a perpetual, paid-in-full license in such country. Baylor may terminate or modify the 2010 Baylor license agreement 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. We may terminate the 2010 Baylor license agreement, or any portion thereof, at our sole discretion at any time upon 60 days' written notice to Baylor. Upon termination of the 2010 Baylor license agreement for any reason prior to expiration, we must assign to Baylor each authorized sublicense agreement that is currently in effect on the date of termination.

#### 2014 Baylor License Agreement

Pursuant to an Exclusive License Agreement with Baylor, effective November 1, 2014, or the 2014 Baylor license agreement, we obtained an exclusive, worldwide license to certain intellectual property, including intellectual property related to methods for inducing selective apoptosis.

Pursuant to the terms of the 2014 Baylor license agreement, we paid Baylor a license execution fee of \$25,000. In addition, we are required to pay Baylor a low annual maintenance fee on each anniversary of the agreement date. The terms of the 2014 Baylor license agreement also require us to make royalty payments in the low single digits, subject to certain annual minimums, on net sales of products covered by the license. To the extent we enter into a sublicensing agreement relating to a licensed product, we are also required to pay Baylor a percentage in the low double-digits on all non-royalty income received from sublicensing revenue. We are required to make milestone payments, of up to \$275,000 in aggregate, upon successful completion of clinical and regulatory milestones regarding the first product covered by this license. The 2014 Baylor license agreement will expire upon expiration of the last patent contained in the licensed patent rights, on a country-by-country basis, upon which we will have a perpetual, paid-in-full license in each such country.

Baylor may terminate or modify the 2014 Baylor license agreement 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. We may terminate the 2014 Baylor license agreement, or any portion thereof, at our sole discretion at any time upon 60 days' written notice to Baylor.

#### Grant Agreement

##### Grant Agreement with Cancer Prevention and Research Institute of Texas

On July 27, 2011, we entered into a Cancer Research Grant Contract, or the Grant Contract, with the Cancer Prevention and Research Institute of Texas, or CPRIT, under which CPRIT awarded a grant not to exceed approximately \$5.7 million to be used by us for the execution of defined clinical development of BPX-501. In addition, CPRIT may award supplemental funding not to exceed ten percent of the total grant amount based upon our progress. To date, we have received approximately \$4.9 million under the grant. The Grant Contract terminated on June 30, 2014, but obligations exist as to licensing, royalty payments, and indemnification provisions.

Pursuant to the Grant Contract, we granted CPRIT with a non-exclusive, irrevocable, royalty-free, perpetual, worldwide license to the intellectual property facilitated by the Grant Contract for and on behalf of CPRIT and other governmental entities and agencies of the State of Texas for education, research and other non-commercial purposes only.

The terms of the Grant Contract require that we pay tiered royalties in the low- to mid-single digit percentages on revenues from sales and licenses of intellectual property facilitated by the Grant Contract. If a third party acquires substantially all of our assets, we have the option to buy out from the royalty obligations by paying a buyout amount that is equal to a percentage of the net grant award proceeds received by us under the Grant Contract, less the aggregate amount of all royalties paid at the time of the buyout. The applicable percentage depends on the timing of the buyout and ranges from 125% to 200%.

We are required to use diligent and commercially reasonable efforts to commercialize or otherwise bring to practical application the results of the funded clinical trial. If CPRIT notifies us of our failure to (1) make the required effort to commercialize any product covered by this agreement or (2) perform our obligations with respect to protection of intellectual property, the rights to any intellectual property and proprietary and confidential information may, at CPRIT's option, revert to CPRIT and CPRIT, at its own cost, can take over the prosecution and maintenance of any impacted patents and commercialize such product candidate. CPRIT's option is subject to our ability to cure any failures identified by CPRIT within 30 days.

## Competition

The biopharmaceutical industry is characterized by intense and dynamic competition to develop new technologies and proprietary therapies. Any product candidates that we successfully develop and commercialize will have to compete with existing therapies and new therapies that may become available in the future. While we believe that our proprietary CID platform, differentiated product candidates and scientific expertise in the field of cellular immunotherapy provide us with competitive advantages, we face potential competition from various sources, including larger and better-funded pharmaceutical, specialty pharmaceutical and biotechnology companies, as well as from academic institutions, governmental agencies and public and private research institutions.

Our lead product candidate, BPX-501, is an adjunct therapy for HSCT with alternative donors that potentially improves stem cell engraftment, accelerates host immune system recovery and treats GvHD. The current standard-of-care that addresses some of the safety challenges associated with HSCT, primarily GvHD, is high-dose steroids. We are aware of other companies that are developing product candidates to improve the outcome of HSCT, including Kiadis Pharma Netherlands B.V. and Molecular Medicine S.p.A.

T-cell based treatments for cancer, such as CAR-T and TCR therapies, have recently been an area of significant research and development by academic institutions and biopharmaceutical companies. BPX-401, BPX-601 and BPX-701 based on our CIDE CAR and Go-CART technologies will compete with product candidates from a number of companies that are currently focused on this therapeutic modality, including Adaptimmune Limited, bluebird bio, Inc., Celgene Corporation, Cellectis SA, GlaxoSmithKline plc, Intrexon Corporation, Juno Therapeutics, Inc., Kite Pharma, Inc., Novartis AG, Pfizer Inc. and Ziopharm Oncology.

BPX-201 based on our DeCIDE technology is a dendritic cell-based cancer vaccine for the treatment of metastatic prostate cancer and other solid tumors. PROVENGE®, marketed by Dendreon Corporation, is the first approved cancer vaccine for the treatment of mCRPC. We are aware of other companies focused on developing cancer vaccines, including Advaxis, Inc., Argos Therapeutics, Inc., Biovest International, Inc., ImmunoCellular Therapeutics, Ltd., Immune Design, Inc., Inovio Pharmaceuticals, Inc., Intrexon Corporation and Northwest Biotherapeutics, Inc.

Many of our competitors, either alone or with their strategic partners, have substantially greater financial, technical and human resources than we do and significantly greater experience in the discovery and development of product

candidates, obtaining FDA and other regulatory approvals of treatments and commercializing those treatments. Accordingly, our competitors may be more successful than us in obtaining approval for treatments and achieving widespread market acceptance. Our competitors' treatments may be more effective, or more effectively marketed and sold, than any treatment we may commercialize and may render our treatments obsolete or non-competitive before we can recover the expenses of developing and commercializing any of our treatments.

Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated among a smaller number of our competitors. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical study sites and subject registration for clinical studies, as well as in acquiring technologies complementary to, or necessary for, our programs. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

We anticipate that we will face intense and increasing competition as new drugs enter the market and advanced technologies become available. We expect any treatments that we develop and commercialize to compete on the basis of, among other things, efficacy, safety, convenience of administration and delivery, price, the level of generic competition and the availability of reimbursement from

government and other third-party payers. For example, if a third party is able to obtain a stand-alone new drug application for rimiducid, then potential generic manufacturers may be able to file abbreviated new drug applications for such product.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we may develop. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. In addition, we expect that our therapeutic products, if approved, will be priced at a significant premium over competitive generic products and our ability to compete may be affected in many cases by insurers or other third-party payers seeking to encourage the use of generic products.

#### Government Regulation and Product Approval

As a biopharmaceutical company that operates in the United States, we are subject to extensive regulation. Our cell products will be regulated as biologics. With this classification, commercial production of our products will need to occur in registered and licensed facilities in compliance with cGMP for biologics.

The FDA regulates human cells, tissues, and cellular and tissue-based products, or HCT/Ps, under a two-tiered framework, based on risk categorization. Higher risk HCT/Ps are regulated as biologics. Manufacturers of biologics are subject to extensive government regulation. For example, such products must complete extensive clinical trials, which must be conducted pursuant to an effective investigational new drug application, or IND. The FDA must review and approve a BLA before a new biologic may be marketed.

The FDA considers our investigational products to be “combination products” because our products involve a biologic (the engineered cells) that is intended to be used with a small molecule chemical drug (AP1903, licensed from ARIAD). In general, biologics such as our engineered cells are regulated through FDA’s Center for Biologics Evaluation and Research, or CBER, while synthetic drugs are regulated through FDA’s Center for Drug Evaluation and Research, or CDER. When FDA encounters a combination product such as our products, the agency determines which of the two centers will have primary responsibility for regulating the product by determining the primary mode of action for the product. In this case, we believe that the cellular component of the combination contributes the primary mode of action and, as a result, that FDA will regulate our investigational products as biologics, through CBER.

Government authorities in the United States (at the federal, state and local level) and in other countries extensively regulate, among other things, the research, development, testing, manufacturing, quality control, approval, labeling, packaging, storage, record-keeping, promotion, advertising, distribution, post-approval monitoring and reporting, marketing and export and import of biopharmaceutical products such as those we are developing. Our product candidates must be approved by the FDA before they may be legally marketed in the United States and by the appropriate foreign regulatory agency before they may be legally marketed in foreign countries. Generally, our activities in other countries will be subject to regulation that is similar in nature and scope as that imposed in the United States, although there can be important differences. Additionally, some significant aspects of regulation in Europe are addressed in a centralized way, but country-specific regulation remains essential in many respects. The process for obtaining regulatory marketing approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources.

#### U.S. Product Development Process

In the United States, the FDA regulates new drugs and biological products under the Federal Food, Drug and Cosmetic Act, or FDCA; the Public Health Service Act, or PHSA; and implementing regulations. Products are also subject to other federal, state and local statutes and regulations. The process of obtaining regulatory approvals and the



subsequent compliance with appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or after approval, may subject an applicant to administrative, criminal, or civil sanctions. FDA sanctions could include, among other actions, refusal to approve pending applications, withdrawal of an approval, a clinical hold, warning letters, product recalls or withdrawals from the market, product seizures, total or partial suspension of production or distribution injunctions, fines, refusals of government contracts, restitution, disgorgement or civil or criminal penalties. Any administrative, criminal, or civil enforcement action could have a material adverse effect on us. The FDA has limited experience with commercial development of T cell therapies for cancer. The process required by the FDA before a biological product may be marketed in the United States generally involves the following:

- completion of nonclinical laboratory tests and animal studies according to good laboratory practices, or GLPs, and applicable requirements for the humane use of laboratory animals or other applicable regulations;
- submission to the FDA of an IND, which must become effective before human clinical trials may begin;

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- performance of adequate and well-controlled human clinical trials according to the FDA's regulations commonly referred to as good clinical practices, or GCPs, and any additional requirements for the protection of human research patients and their health information, to establish the safety and efficacy of the proposed biological product for its intended use;
- submission to the FDA of a BLA for marketing approval that includes substantial evidence of safety, purity, and potency from results of nonclinical testing and clinical trials;
- satisfactory completion of an FDA inspection of the manufacturing facility or facilities where the biological product is produced to assess compliance with cGMP, to assure that the facilities, methods and controls are adequate to preserve the biological product's identity, strength, quality and purity and, if applicable, the FDA's current good tissue practices, or GTPs, for the use of HCT/Ps;
- potential FDA audit of the nonclinical study and clinical trial sites that generated the data in support of the BLA; and
- FDA review and approval, or licensure, of the BLA.

Before testing any biological product candidate, including our product candidates, in humans, the product candidate enters the preclinical testing stage. Preclinical tests, also referred to as nonclinical studies, include laboratory evaluations of product chemistry, toxicity and formulation, as well as animal studies to assess the potential safety and activity of the product candidate. The conduct of the preclinical tests must comply with federal regulations and requirements including GLPs. The clinical trial sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and a proposed clinical protocol, to the FDA as part of the IND. Some preclinical testing may continue even after the IND is submitted. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA raises concerns or questions regarding the proposed clinical trials and places the trial on a clinical hold within that 30-day time period. In such a case, the IND sponsor must resolve FDA's outstanding concerns before the clinical trial can begin. The FDA may also impose clinical holds on a biological product candidate at any time before or during clinical trials due to safety concerns or non-compliance. If the FDA imposes a clinical hold, trials may not recommence without FDA authorization and then only under terms authorized by the FDA. Accordingly, we cannot be sure that submission of an IND will result in the FDA allowing clinical trials to begin, or that, once begun, issues will not arise that suspend or terminate such clinical trials.

Clinical trials involve the administration of the biological product candidate to healthy volunteers or patients under the supervision of qualified investigators, generally physicians not employed by or under the trial sponsor's control. Clinical trials are conducted under protocols detailing, among other things, the objectives of the clinical trial, dosing procedures, subject selection and exclusion criteria, and the parameters to be used to monitor subject safety, including stopping rules that assure a clinical trial will be stopped if certain adverse events should occur. Each protocol and any amendments to the protocol must be submitted to the FDA as part of the IND. Clinical trials must be conducted and monitored in accordance with the FDA's regulations comprising the GCP requirements, including the requirement that all research patients provide informed consent. Further, each clinical trial must be reviewed and approved by an institutional review board, or IRB, at or servicing each institution at which the clinical trial will be conducted. An IRB is independent from the trial sponsor and is charged with protecting the welfare and rights of clinical trial participants and considers such items as whether the risks to individuals participating in the clinical trials are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the form and content of the informed consent that must be signed by each clinical trial subject or his or her legal representative and must monitor the clinical trial until completed. Clinical trials also must be reviewed by an institutional biosafety committee, or IBC, a local institutional committee that reviews and oversees basic and clinical research conducted at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment.

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

- Phase 1. The biological product is initially introduced into healthy human subjects and tested for safety. In the case of some products for severe or life-threatening diseases, especially when the product may be too inherently toxic to ethically administer to healthy volunteers, the initial human testing is often conducted in patients.

·Phase 2. The biological product is evaluated in a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance, optimal dosage and dosing schedule.

·Phase 3. Clinical trials are undertaken to further evaluate dosage, clinical efficacy, potency, and safety in an expanded patient population at geographically dispersed clinical trial sites. These clinical trials are intended to establish the overall risk to benefit ratio of the product and provide an adequate basis for product labeling.

Post-approval clinical trials, sometimes referred to as Phase 4 clinical trials, may be conducted after initial marketing approval. These clinical trials are used to gain additional experience from the treatment of patients in the intended therapeutic indication, particularly for long-term safety follow-up.

During all phases of clinical development, regulatory agencies require extensive monitoring and auditing of all clinical activities, clinical data, and clinical trial investigators. Annual progress reports detailing the progress of the clinical trials must be submitted to the FDA. Written IND safety reports must be promptly submitted to the FDA, the NIH and the investigators for serious and unexpected adverse events, any findings from other studies, tests in laboratory animals or in vitro testing that suggest a significant risk for human patients, or any clinically important increase in the rate of a serious suspected adverse reaction over that listed in the protocol or investigator brochure. The sponsor must submit an IND safety report within 15 calendar days after the sponsor determines that the information qualifies for reporting. The sponsor also must notify the FDA of any unexpected fatal or life-threatening suspected adverse reaction within seven calendar days after the sponsor's initial receipt of the information. Phase 1, Phase 2 and Phase 3 clinical trials may not be completed successfully within any specified period, if at all. The FDA or the sponsor or its data safety monitoring board may suspend or terminate a clinical trial at any time on various grounds, including a finding that the research patients are being exposed to an unacceptable health risk, including risks inferred from other unrelated immunotherapy trials. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the biological product has been associated with unexpected serious harm to patients.

Human immunotherapy products are a new category of therapeutics. Because this is a relatively new and expanding area of novel therapeutic interventions, there can be no assurance as to the length of the trial period, the number of patients the FDA will require to be enrolled in the clinical trials in order to establish the safety, efficacy, purity and potency of immunotherapy products, or that the data generated in these clinical trials will be acceptable to the FDA to support marketing approval.

Concurrently with clinical trials, companies usually complete additional studies and must also develop additional information about the physical characteristics of the biological product as well as finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. To help reduce the risk of the introduction of adventitious agents with use of biological products, the PHSA emphasizes the importance of manufacturing control for products whose attributes cannot be precisely defined. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, the sponsor must develop methods for testing the identity, strength, quality, potency and purity of the final biological product. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the biological product candidate does not undergo unacceptable deterioration over its shelf life.

Federal law requires that we register all of our clinical trials on a publicly accessible website. We must also provide results information for most of our clinical trials, other than Phase 1 clinical trials.

#### U.S. Review and Approval Processes

After the completion of clinical trials of a biological product, FDA approval of a BLA must be obtained before commercial marketing of the biological product. The BLA must include results of product development, laboratory and animal studies, human trials, information on the manufacture and composition of the product, proposed labeling and other relevant information. The FDA may grant deferrals for submission of certain data or full or partial waivers. The testing and approval processes require substantial time and effort and there can be no assurance that the FDA will accept the BLA for filing and, even if filed, that any approval will be granted on a timely basis, if at all.

Under the Prescription Drug User Fee Act, or PDUFA, as amended, each BLA must be accompanied by a significant user fee. The FDA adjusts the PDUFA user fees on an annual basis. PDUFA also imposes an annual product fee for biological products and an annual establishment fee on facilities used to manufacture prescription biological products. Fee waivers or reductions are available in certain circumstances, including a waiver of the application fee for the first application filed by a small business. Additionally, no user fees are assessed on BLAs for products designated as orphan drugs, unless the application also includes a non-orphan indication.

Within 60 days following submission of the application, the FDA reviews a BLA submitted to determine if it is substantially complete before the agency accepts it for filing. The FDA may refuse to file any BLA that it deems incomplete or not properly reviewable at the time of submission and may request additional information. In this event, the BLA must be resubmitted with the additional information. The resubmitted application also is subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review of the BLA. The FDA reviews the BLA to determine, among other things, whether the proposed product is safe, potent, and/or effective for its intended use, and has an acceptable purity profile, and whether the product is being manufactured in accordance with cGMP to assure and preserve the product's identity, safety, strength, quality, potency and purity. The FDA may refer applications for novel biological products or biological products that present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions. During the biological product approval process, the FDA also will determine whether a Risk Evaluation and Mitigation Strategy, or REMS, is necessary to assure the safe use of the biological product. If the FDA concludes a REMS is needed, the sponsor of the BLA must submit a proposed REMS. The FDA will not approve a BLA without a REMS, if required.

Before approving a BLA, the FDA will inspect the facilities at which the product is manufactured. The FDA will not approve the product unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. For immunotherapy products, the FDA also will not approve the product if the manufacturer is not in compliance with the GTPs, to the extent applicable. These are FDA regulations and guidance documents that govern the methods used in, and the facilities and controls used for, the manufacture of HCT/Ps. The primary intent of the GTP requirements is to ensure that cell and tissue based products are manufactured in a manner designed to prevent the introduction, transmission and spread of communicable disease. FDA regulations also require HCT/P establishments to register and list their HCT/Ps with the FDA and, when applicable, to evaluate donors through screening and testing. Additionally, before approving a BLA, the FDA will typically inspect one or more clinical sites to assure that the clinical trials were conducted in compliance with IND trial requirements and GCP requirements. To maintain compliance with cGMPs, GTPs, and GCPs, an applicant must incur significant expenditure of time, money and effort in the areas of training, record keeping, production, and quality control.

Notwithstanding the submission of relevant data and information, the FDA may ultimately decide that the BLA does not satisfy its regulatory criteria for approval and deny approval. Data obtained from clinical trials are not always conclusive and the FDA may interpret data differently than we interpret the same data. If the agency decides not to approve the BLA in its present form, the FDA will issue a complete response letter that describes all of the specific deficiencies in the BLA identified by the FDA. The deficiencies identified may be minor, for example, requiring labeling changes, or major, for example, requiring additional clinical trials. Additionally, the complete response letter may include recommended actions that the applicant might take to place the application in a condition for approval. If a complete response letter is issued, the applicant may either resubmit the BLA, addressing all of the deficiencies identified in the letter, or withdraw the application.

If a product receives regulatory approval, the approval may be significantly limited to specific diseases and dosages or the indications for use may otherwise be limited, which could restrict the commercial value of the product.

Further, the FDA may require that certain contraindications, warnings or precautions be included in the product labeling. The FDA may impose restrictions and conditions on product distribution, prescribing, or dispensing in the form of a REMS or other risk management plan, or otherwise limit the scope of any approval. In addition, the FDA may require post marketing clinical trials, sometimes referred to as Phase 4 clinical trials, designed to further assess a biological product's safety and effectiveness, and testing and surveillance programs to monitor the safety of approved products that have been commercialized.

In addition, under the Pediatric Research Equity Act, or PREA, a BLA or supplement to a BLA must contain data to assess the safety and effectiveness of the product for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may grant deferrals for submission of data or full or partial waivers. Unless otherwise required by regulation, PREA does not apply to any product for an indication for which orphan designation has been granted. However, if only one indication for a product has orphan designation, a pediatric assessment may still be required for any applications to market that same product for the non-orphan indication(s). Sponsors in satisfaction of this obligation may receive an additional six months of marketing exclusivity for all dosage forms and all indications with the same active moiety as the drug studied.

#### Orphan Drug Designation

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug or biologic intended to treat a rare disease or condition, which is generally a disease or condition that affects fewer than 200,000 individuals in the United States, or more than 200,000 individuals in the United States and for which there is no reasonable expectation that the cost of developing and making available in the United States a drug or biologic for this type of disease or condition will be recovered from sales in the United States for that drug or biologic. Orphan drug designation must be

requested before submitting a BLA. After the FDA grants orphan drug designation, the generic identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. The orphan drug designation does not shorten the duration of the regulatory review or approval process, but does provide certain advantages, such as a waiver of PDUFA fees, enhanced access to FDA staff, and potential waiver of PREA requirements discussed above.

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 full 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. Orphan drug exclusivity does not prevent FDA from approving a different drug or biologic for the same disease or condition, or the same drug or biologic for a different disease or condition. Among the other benefits of orphan drug designation are tax credits for certain research and a waiver of the BLA application user fee.

A designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation. In addition, exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition.

We are currently in discussions with FDA regarding orphan drug designation for our investigational products.

#### Expedited Development and Review Programs

The FDA has a Fast Track program that is intended to expedite or facilitate the process for reviewing new products that meet certain criteria. Specifically, new products are eligible for Fast Track designation if they are intended to treat a serious or life-threatening disease or condition and demonstrate the potential to address unmet medical needs, or if the drug has been designated as a qualified infectious disease product. Fast Track designation applies to the combination of the product and the specific indication for which it is being studied. Under Fast Track, the FDA may consider for review sections of the BLA on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the BLA, the FDA agrees to accept sections of the BLA and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the BLA. Even if Fast Track designation is granted, it may be rescinded if the product no longer meets the qualifying criteria.

Any product, submitted to the FDA for approval, including a product with a Fast Track designation, may also be eligible for other types of FDA programs intended to expedite development and review, such as priority review and accelerated approval. A product is eligible for priority review if it treats a serious condition and, if approved, would provide a significant improvement in safety and effective. The FDA will attempt to direct additional resources to the evaluation of an application for a new product designated for priority review in an effort to facilitate the review. Additionally, a product may be eligible for accelerated approval. Products studied for their safety and effectiveness in treating serious or life-threatening diseases or conditions may receive accelerated approval upon a determination that the product treats a serious condition, provides a meaningful advantage over available therapies, and demonstrates an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. As a condition of approval, the FDA may require that a sponsor of a drug or biological product receiving accelerated approval perform appropriate post-marketing clinical studies to verify and describe the predicted effect on irreversible morbidity or mortality or other clinical benefit. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials, which could adversely impact the timing of the commercial launch of the product. The FDCA also provides expedited procedures for FDA withdrawal of approval of a product approved through accelerated approval. Fast Track designation, priority review and accelerated approval do not change the standards for approval but may expedite the development or approval process.

In 2012 the FDA established a Breakthrough Therapy Designation which is intended to expedite the development and review of products that treat serious or life-threatening conditions. The designation requires preliminary clinical evidence that may demonstrate substantial improvement on a clinically significant endpoint over available therapies. The designation includes all of the Fast Track program features, as well as more intensive FDA interaction and guidance, organizational commitment, and other potential actions to expedite review. The Breakthrough Therapy Designation is a distinct status from both accelerated approval and priority review, which can also be granted to the same product if relevant criteria are met. If a product is designated as breakthrough therapy, FDA will expedite the development and review of such product. Even if a Breakthrough Therapy Designation is granted, it may be rescinded if the product no longer meets the qualifying criteria.

Where applicable, we plan to request Fast Track and Breakthrough Therapy Designation for our product candidates, including BPX-051, BPX-401 and BPX-601. Even if we receive one or both of these designations for our product candidates, the FDA may later decide that our product candidates no longer meets the conditions for qualification. In addition, these designations may not provide us with a material commercial advantage.

#### Post-Approval Requirements



Any product for which we receive FDA approval is subject to continuing regulation by the FDA, including, among other things, record-keeping requirements, reporting of adverse experiences with the product, providing the FDA with updated safety and efficacy information, product sampling and distribution requirements, and complying with FDA promotion and advertising requirements, which include, among others, standards for direct-to-consumer advertising, restrictions on promoting products for uses or in patient populations that are not described in the product's approved uses (known as "off-label use"), limitations on industry-sponsored scientific and educational activities, and requirements for promotional activities involving the internet. Although physicians may prescribe legally available products for off-label uses, if the physicians deem it to be appropriate in their professional medical judgment, manufacturers may not market or promote such off-label uses.

In addition, quality control and manufacturing procedures must continue to conform to applicable manufacturing requirements after approval to ensure the long-term stability of the product. We rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of our products in accordance with cGMP regulations. cGMP regulations require among other

things, quality control and quality assurance as well as the corresponding maintenance of records and documentation and the obligation to investigate and correct any deviations from cGMP. Manufacturers and other entities involved in the manufacture and distribution of approved products are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMP and other laws. Accordingly, manufacturers must continue to expend time, money, and effort in the area of production and quality control to maintain cGMP compliance. Discovery of problems with a product after approval may result in restrictions on a product, manufacturer, or holder of an approved BLA, including, among other things, recall or withdrawal of the product from the market, seizure of product manufactured not in accordance with GMPs, suspension or termination of manufacturing activities at one or more facilities, or other civil or criminal sanctions. In addition, changes to the manufacturing process are strictly regulated, and depending on the significance of the change, may require prior FDA approval before being implemented. Other types of changes to the approved product, such as adding new indications and claims, are also subject to further FDA review and approval.

The FDA also may require post-marketing testing, known as Phase 4 testing, and surveillance to monitor the effects of an approved product. Discovery of previously unknown problems with a product or the failure to comply with applicable FDA requirements can have negative consequences, including adverse publicity, judicial or administrative enforcement, warning letters from the FDA, mandated corrective advertising or communications with doctors, and civil or criminal penalties, among others. Newly discovered or developed safety or effectiveness data may require changes to a product's approved labeling, including the addition of new warnings and contraindications, and also may require the implementation of a REMS or other risk management measures. Also, new government requirements, including those resulting from new legislation, may be established, or the FDA's policies may change, which could delay or prevent regulatory approval of our products under development.

#### U.S. Patent Term Restoration and Marketing Exclusivity

The Biologics Price Competition and Innovation Act, or BPCIA, amended the PHSA to authorize the FDA to approve similar versions of innovative biologics, commonly known as biosimilars. Among other requirements, a competitor seeking approval of a biosimilar must file an application to establish its molecule as highly similar to an approved innovator biologic, without any clinically meaningful differences in terms of safety, purity, and potency. The BPCIA, however, bars the FDA from approving biosimilar applications for 12 years after an innovator biological product receives initial marketing approval. This 12-year period of data exclusivity may be extended by six months, for a total of 12.5 years, if the FDA requests that the innovator company conduct pediatric clinical investigations of the product. Although a statutory provision exists for FDA approval of biosimilars, FDA has yet to provide clarity on many aspects of the regulatory pathway for such products. Furthermore, the first biosimilar applications have only recently been submitted to FDA, and it remains to be seen how FDA will apply the statutory biosimilar provisions to biological products such as ours.

Depending upon the timing, duration and specifics of the FDA approval of the use of our product candidates, some of our U.S. patents, if granted, may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, commonly referred to as the Hatch-Waxman Act. The Hatch-Waxman Act permits a patent restoration term of up to five years, as compensation for patent term lost during product development and the FDA regulatory review process. However, patent term restoration cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date. The patent term restoration period is generally one-half the time between the effective date of an IND and the submission date of a BLA plus the time between the submission date of a BLA and the approval of that application. Only one patent applicable to an approved product is eligible for the extension and the application for the extension must be submitted prior to the expiration of the patent. The U.S. Patent and Trademark Office, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, we may intend to apply for restoration of patent term for one of our currently owned or licensed patents to add patent life beyond its current expiration date, depending on the expected length of the clinical trials and other factors involved in the filing of the relevant BLA.

Other U.S. Healthcare Laws and Compliance Requirements

In the United States, our activities are potentially subject to regulation by various federal, state and local authorities in addition to the FDA, including but not limited to, the Centers for Medicare and Medicaid Services, or CMS, other divisions of the U.S. Department of Health and Human Services (e.g., the Office of Inspector General), the U.S. Department of Justice, or DOJ, and individual U.S. Attorney offices within the DOJ, and state and local governments. For example, sales, marketing and scientific/educational grant programs must comply with the anti-fraud and abuse provisions of the Social Security Act, the false claims laws, the privacy provisions of the Health Insurance Portability and Accountability Act, or HIPAA, the sunshine provisions of the Affordable Care Act, and similar state laws, each as amended.

The federal Anti-Kickback Statute prohibits, among other things, any person or entity, from knowingly and willfully offering, paying, soliciting or receiving any remuneration, directly or indirectly, overtly or covertly, in cash or in kind, to induce or in return for purchasing, leasing, ordering or arranging for the purchase, lease or order of any good, facility, item or service reimbursable, in whole

or in part, under Medicare, Medicaid or other federal healthcare programs. The term remuneration has been interpreted broadly to include anything of value. The Anti-Kickback Statute has been interpreted to apply to arrangements between biologic manufacturers on one hand and prescribers, purchasers, and formulary managers on the other. There are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution. The exceptions and safe harbors are drawn narrowly and practices that involve remuneration that may be alleged to be intended to induce prescribing, purchasing or recommending may be subject to scrutiny if they do not qualify for an exception or safe harbor. Failure to meet all of the requirements of a particular applicable statutory exception or regulatory safe harbor does not make the conduct per se illegal under the Anti-Kickback Statute. Instead, the legality of the arrangement will be evaluated on a case-by-case basis based on a cumulative review of all of its facts and circumstances.

Additionally, the intent standard under the Anti-Kickback Statute was amended by the Affordable Care Act to a stricter standard such that a person or entity no longer needs to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation. In addition, the Affordable Care Act codified case law that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the federal civil False Claims Act.

The civil monetary penalties statute imposes penalties against any person or entity who, among other things, is determined to have presented or caused to be presented a claim to a federal health program that the person knows or should know is for an item or service that was not provided as claimed or is false or fraudulent.

The federal false claims laws, including but not limited to the federal civil False Claims Act, prohibit, among other things, any person or entity from knowingly presenting, or causing to be presented, a false claim for payment to, or approval by, the federal government. Pharmaceutical and other healthcare companies have been prosecuted under these laws for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product. Other companies have been prosecuted for causing false claims to be submitted because of the companies' marketing of the product for unapproved (i.e., off-label), and thus non-reimbursable, uses.

HIPAA created new federal criminal statutes that prohibit knowingly and willfully executing, or attempting to execute, a scheme to defraud or to obtain, by means of false or fraudulent pretenses, representations or promises, any money or property owned by, or under the control or custody of, any healthcare benefit program, including private third-party payors and knowingly and willfully falsifying, concealing or covering up by trick, scheme or device, a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services.

Also, many states have similar fraud and abuse statutes or regulations that apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payor.

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

Additionally, the federal Physician Payments Sunshine Act within the Affordable Care Act, and its implementing regulations, require that certain manufacturers of drugs, devices, biological and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) to report annually information related to certain payments or other transfers of value made or distributed to physicians and teaching hospitals, or to entities or individuals at the request of, or designated on behalf of, the physicians and teaching hospitals and to report annually certain ownership and investment interests held by physicians and their immediate family members.

We will also be required to begin satisfying the product tracing, verification, and reporting requirements set out in the newly enacted Drug Quality and Security Act.

In order to distribute products commercially, we must also comply with state laws that require the registration of manufacturers and wholesale distributors of drug and biological products in a state, including, in certain states, manufacturers and distributors who ship products into the state even if such manufacturers or distributors have no place of business within the state.

Several states have enacted legislation requiring pharmaceutical and biotechnology companies to, among other things, establish marketing compliance programs, file periodic reports with the state, make periodic public disclosures on sales, marketing, pricing, clinical trials and other activities, and/or register their sales representatives, as well as to prohibit pharmacies and other healthcare entities from providing certain physician prescribing data to pharmaceutical and biotechnology companies for use in sales and marketing, and to prohibit certain other sales and marketing practices. All of our activities are potentially subject to federal and state consumer protection and unfair competition laws.

If our operations are found to be in violation of any of the federal and state healthcare laws described above or any other governmental regulations that apply to us, we may be subject to penalties, including without limitation, civil, criminal and/or administrative penalties, damages, fines, disgorgement, exclusion from participation in government programs, such as Medicare and Medicaid, injunctions, private “qui tam” actions brought by individual whistleblowers in the name of the government, or refusal to allow us to enter into government contracts, contractual damages, reputational harm, administrative burdens, diminished profits and future earnings, and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

### Coverage, Pricing and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any product candidates for which we obtain regulatory approval. In the United States and markets in other countries, sales of any products for which we receive regulatory approval for commercial sale will depend, in part, on the extent to which third-party payors provide coverage and establish adequate reimbursement levels for such products. In the United States, third-party payors include federal and state healthcare programs, private managed care providers, health insurers and other organizations. The process for determining whether a third-party payor will provide coverage for a product may be separate from the process for setting the price of a product or for establishing the reimbursement rate that such a payor will pay for the product. Third-party payors may limit coverage to specific products on an approved list, also known as a formulary, which might not include all of the FDA-approved products for a particular indication. Third-party payors are increasingly challenging the price, examining the medical necessity and reviewing the cost-effectiveness of medical products, therapies and services, in addition to questioning their safety and efficacy. We may need to conduct expensive pharmaco-economic studies in order to demonstrate the medical necessity and cost-effectiveness of our products, in addition to the costs required to obtain the FDA approvals. Our product candidates may not be considered medically necessary or cost-effective. A payor’s decision to provide coverage for a product does not imply that an adequate reimbursement rate will be approved. Further, one payor’s determination to provide coverage for a product does not assure that other payors will also provide coverage for the product. Adequate third-party reimbursement may not be available to enable us to maintain price levels sufficient to realize an appropriate return on our investment in product development.

Different pricing and reimbursement schemes exist in other countries. In the EU, governments influence the price of pharmaceutical products through their pricing and reimbursement rules and control of national health care systems that fund a large part of the cost of those products to consumers. Some jurisdictions operate positive and negative list systems under which products may only be marketed once a reimbursement price has been agreed. To obtain reimbursement or pricing approval, some of these countries may require the completion of clinical trials that compare the cost-effectiveness of a particular product candidate to currently available therapies. Other member states allow companies to fix their own prices for medicines, but monitor and control company profits. The downward pressure on health care costs has become very intense. As a result, increasingly high barriers are being erected to the entry of new products. In addition, in some countries, cross-border imports from low-priced markets exert a commercial pressure on pricing within a country.

The marketability of any product candidates for which we receive regulatory approval for commercial sale may suffer if the government and third-party payors fail to provide adequate coverage and reimbursement. In addition, emphasis on managed care in the United States has increased and we expect will continue to increase the pressure on healthcare

pricing. Coverage policies and third-party reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

#### Healthcare Reform

In March 2010, President Obama signed the Affordable Care Act, which has the potential to substantially change healthcare financing and delivery by both governmental and private insurers, and significantly impact the pharmaceutical and biotechnology industry. The Affordable Care Act will impact existing government healthcare programs and will result in the development of new programs.

Among the Affordable Care Act's provisions of importance to the pharmaceutical and biotechnology industries, in addition to those otherwise described above, are the following:

- an annual, nondeductible fee on any entity that manufactures or imports certain specified branded prescription drugs and biologic agents apportioned among these entities according to their market share in some government healthcare programs, that began in 2011;
- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program, retroactive to January 1, 2010, to 23.1% and 13% of the average manufacturer price for most branded and generic drugs, respectively and capped the total rebate amount for innovator drugs at 100% of the Average Manufacturer Price, or AMP;
- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturers' outpatient drugs to be covered under Medicare Part D;
- extension of manufacturers' Medicaid rebate liability to covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations;
- expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to additional individuals with income at or below 133% of the federal poverty level, thereby potentially increasing manufacturers' Medicaid rebate liability;
- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program; and
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

We anticipate that the Affordable Care Act will result in additional downward pressure on coverage and the price that we receive for any approved product, and could seriously harm our business. Any reduction in reimbursement from Medicare and other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our products.

There have also been changes to the reimbursement landscape in the U.S. since the passage of the Affordable Care Act. On August 2, 2011, the President signed into law the Budget Control Act of 2011, which, among other things, created the Joint Select Committee on Deficit Reduction to recommend to Congress proposals in spending reductions. The Joint Select Committee did not achieve a targeted deficit reduction of at least \$1.2 trillion for the years 2013 through 2021, triggering the legislation's automatic reduction to several government programs. This includes reductions to Medicare payments to providers of 2% per fiscal year, which went into effect on April 1, 2013 and will stay in effect through 2024 unless additional Congressional action is taken. On January 2, 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, reduced Medicare payments to several providers, 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. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our products and/or additional pricing pressure. In addition, it is possible that there will be further legislation or regulation that could harm our business, financial condition, and results of operations.

#### The Foreign Corrupt Practices Act

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





### Additional Regulation

In addition to the foregoing, state and federal laws regarding environmental protection and hazardous substances, including the Occupational Safety and Health Act, the Resource Conservancy and Recovery Act and the Toxic Substances Control Act, affect our business. These and other laws govern our use, handling and disposal of various biological, chemical and radioactive substances used in, and wastes generated by, our operations. If our operations result in contamination of the environment or expose individuals to hazardous substances, we could be liable for damages and governmental fines. We believe that we are in material compliance with applicable environmental laws and that continued compliance therewith will not have a material adverse effect on our business. We cannot predict, however, how changes in these laws may affect our future operations.

### Europe / Rest of World Government Regulation

In addition to regulations in the United States, we will be subject to a variety of regulations in other jurisdictions governing, among other things, clinical trials and any commercial sales and distribution of our products. Whether or not we obtain FDA approval of a product, we must obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical trials or marketing of the product in those countries. Certain countries outside of the United States have a similar process that requires the submission of a clinical trial application much like the IND prior to the commencement of human clinical trials. In the EU, for example, a clinical trial application must be submitted to each country's national health authority and an independent ethics committee, much like the FDA and IRB, respectively. Once the clinical trial application is approved in accordance with a country's requirements, clinical trial development may proceed. Because biologically sourced raw materials are subject to unique contamination risks, their use may be restricted in some countries. The requirements and process governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. In all cases, the clinical trials are conducted in accordance with GCP and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

To obtain regulatory approval of an investigational drug or biological product under EU regulatory systems, we must submit a marketing authorization application. The application used to file the BLA in the United States is similar to that required in the EU, with the exception of, among other things, country-specific document requirements.

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

### Employees

As of December 31, 2014, we had 35 employees, all of whom were full-time, 27 of whom were engaged in research and development activities and 8 of whom were engaged in business development, finance, information systems, facilities, human resources or administrative support. None of our employees is subject to a collective bargaining agreement. We consider our relationship with our employees to be good.

### 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](http://www.bellicum.com). Our Annual Report on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K, and amendments to reports filed pursuant to Sections 13(a) and 15(d) of the Securities Exchange Act of 1934, as amended, or the Exchange Act, will be made available free of charge on our website as soon as reasonably practicable after we electronically file such material with, or furnish it to, the Securities and Exchange Commission, or

the SEC. The contents of our website are not incorporated into this Annual Report and our reference to the URL for our website is intended to be an inactive textual reference only.

We are an “emerging growth company,” as defined in the JOBS Act. 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 our initial public offering in December 2014, (b) in which we have total annual gross revenue of at least \$1.0 billion, or (c) in which we are deemed to be a large accelerated filer, which means the market value of our common stock that is held by non-affiliates exceeded \$700.0 million as of the prior June 30th, and (2) the date on which we have issued more than \$1.0 billion in non-convertible debt during the prior three-year period. References to “emerging growth company” in this Annual Report on Form 10-K have the meaning associated with it in the JOBS Act.

## ITEM 1A. Risk Factors

You should carefully consider the following risk factors, as well as the other information in this report, and in our other public filings. The occurrence of any of the following risks could harm our business, financial condition, results of operations and/or growth prospects or cause our actual results to differ materially from those contained in forward-looking statements we have made in this report and those we may make from time to time. You should consider all of the risk factors described when evaluating our business.

### 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 equity and debt financings. For the years ended December 31, 2014 and 2013, we reported a net loss of \$84.0 million and \$8.0 million, respectively. As of December 31, 2014, we had an accumulated deficit of \$112.9 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:

- 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;
- seeking and obtaining marketing approvals for product candidates that successfully complete clinical trials;
- 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;
- identifying and developing new product candidates;
- progressing our pre-clinical programs into human clinical trials;
- establishing and maintaining supply and manufacturing relationships with third parties;
- developing new molecular switches based on our proprietary CID technology platform;
- maintaining, protecting, expanding and enforcing our intellectual property; and
- attracting, hiring and retaining qualified personnel.

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 stockholders to lose all or part of their 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:

- obtaining regulatory approval, as the FDA and other regulatory authorities have limited experience with commercial development of T-cell therapies for cancer;
- sourcing clinical and, if approved, commercial supplies for the materials used to manufacture and process our product candidates;
- 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;
- educating medical personnel regarding the potential safety benefits, as well as the challenges, of incorporating our product candidates into their treatment regimens; and
- 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, and 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, 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 clinical trials of BPX-501 primarily to assess safety and efficacy in a small number of patients with malignant disease or inherited blood disorders. 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. 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.

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:

- the patient eligibility criteria defined in the protocol;
- the size of the patient population required for analysis of the trial's primary endpoints;
- the proximity of patients to study sites;
- the design of the clinical trial;
- our ability to recruit clinical trial investigators with the appropriate competencies and experience;
- our ability to obtain and maintain patient consents; and
- 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 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, Cellectis SA, GlaxoSmithKline plc, Intrexon Corporation, Juno Therapeutics, Inc., Kite Pharma, Inc., Novartis AG, Pfizer Inc. and Ziopharm Oncology. Our lead product candidate, BPX-501, is an adjunct therapy for HSCT with alternative donors that potentially improves stem cell engraftment, accelerates host immune system recovery and treats GvHD. Other companies that we are aware of that are developing product candidates to improve the outcome of HSCT, include Kiadis Pharma Netherlands B.V. and Molecular Medicine S.p.A. . BPX-201, based on our DeCIDE technology, is a dendritic cell-based cancer vaccine for the treatment of metastatic prostate cancer and other solid tumors. Other companies that we are aware of that are focused on developing cancer vaccines, include Advaxis, Inc., Argos Therapeutics, Inc., Biovest International, Inc., ImmunoCellular Therapeutics, Ltd., Immune Design, Inc., Inovio Pharmaceuticals, Inc., Intrexon Corporation and Northwest Biotherapeutics, 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 "Item 1. Business—Competition."

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 Financial 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 December 31, 2014, we had 35 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:

- identifying, recruiting, integrating, maintaining and motivating additional employees;

- 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
- 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 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 December 31, 2014, we had cash and cash equivalents of approximately \$191.6 million, which included the net proceeds from our recently completed initial public offering. We believe that such cash and cash equivalents will be sufficient to fund our operations through at least the first half of 2017. 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, the ownership interests of our existing stockholders will be diluted, and the terms may include liquidation or other preferences that adversely affect the rights of our stockholders. 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.

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:

- 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.
- 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.
- Contract manufacturers may not be able to execute our manufacturing procedures appropriately.
- 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.
- 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.
- 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.
- 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 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.

We may not 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:

- differing regulatory requirements in foreign countries;
- unexpected changes in tariffs, trade barriers, price and exchange controls and other regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign taxes, including withholding of payroll taxes;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country;
- difficulties staffing and managing foreign operations;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- potential liability under the Foreign Corrupt Practices Act of 1977 or comparable foreign regulations;
- 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;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- 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 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.

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:

- 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;

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- 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;
- 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;
- 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;
- the federal Physician Payment Sunshine Act, created under the Health Reform Law, and its implementing regulations, which requires certain 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
- 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.

We have adopted 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:

- decreased demand for our product candidates;
- injury to our reputation;
- withdrawal of clinical trial participants;

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- initiation of investigations by regulators;
- costs to defend the related litigation;
- a diversion of management's time and our resources;
- substantial monetary awards to clinical trial participants or patients;
- product recalls, withdrawals or labeling, marketing or promotional restrictions;
- loss of revenue;
- exhaustion of any available insurance and our capital resources;
- the inability to commercialize any product candidate; and
- 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 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 recently completed initial public offering, our most recent private placements and other transactions that have occurred over the past three years, we may have experienced 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, 2014, we had U.S. net operating loss carryforwards of approximately \$41.3 million, which begin to expire in 2024, and U.S. research and development credits of \$1.3 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:

- the availability of financial resources to commence and complete our planned clinical trials;
- 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;
- obtaining approval at each clinical trial site by an independent institutional review board, or IRB;
- recruiting suitable patients to participate in a clinical trial;
- having patients complete a clinical trial or return for post-treatment follow-up;

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- clinical trial sites deviating from clinical trial protocol, failing to follow GCPs, or dropping out of a clinical trial;
- adding new clinical trial sites; or
- 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 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:

- the FDA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials;
- we may be unable to demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities that our product candidates have the necessary safety, purity, and potency for any of their proposed indications;
- the results of clinical trials may not meet the level of statistical significance required by the FDA or comparable foreign regulatory authorities for approval;
- we may be unable to demonstrate that our product candidates' clinical and other benefits outweigh their safety risks;
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- we may encounter serious and unexpected adverse events during clinical trials that render our products unsafe for use in humans;
- the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from preclinical studies or clinical trials;
- 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;

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- 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
- 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.

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:

- restrictions on the marketing or manufacturing of our product candidates, withdrawal of the product from the market, or voluntary or mandatory product recalls;
- fines, warning letters or holds on clinical trials;
- refusal by the FDA to approve pending applications or supplements to approved applications filed by us or suspension or revocation of license approvals;



- suspension or termination of manufacturing at one or more manufacturing facilities;
- product seizure or detention, or refusal to permit the import or export of our product candidates; and
- 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.

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:

- the clinical indications for which our product candidates are approved;
- physicians, hospitals, cancer treatment centers and patients considering our product candidates as a safe and effective treatment;
- the potential and perceived advantages of our product candidates over alternative treatments;
- the prevalence and severity of any side effects;
- product labeling or product insert requirements of the FDA or other regulatory authorities;
- limitations or warnings contained in the labeling approved by the FDA;
- the timing of market introduction of our product candidates as well as competitive products;
- the cost of treatment in relation to alternative treatments;
- the availability of adequate coverage, reimbursement and pricing by third-party payors and government authorities;
- the willingness of patients to pay out-of-pocket in the absence of coverage by third-party payors, including government authorities;
- relative convenience and ease of administration, including as compared to alternative treatments and competitive therapies;
- confusion or lack of understanding regarding the effects of rimiducid and the timing and size of dosing of rimiducid after immune cell therapy; and
- 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 are currently discussing the designation of orphan for the combination of BPX-501 and rimiducid for treatment of immunodeficiency after allogeneic transplant

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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:

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

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

#### 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 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.

Any termination of these agreements could result in the loss of significant rights and could harm our ability to commercialize our product candidates. See “Item 1. 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:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- 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;
- our right to sublicense patent and other rights to third parties under collaborative development relationships;
- 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
- 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. 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.

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:

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

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 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 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.

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## Risks Related to Ownership of our Common Stock

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

Prior to our recently completed initial public offering, there was no public market for our common stock. The trading price of our common stock 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 Annual Report, these factors include:

- 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;
- 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;
- adverse results or delays in clinical trials;
- our decision to initiate a clinical trial, not to initiate a clinical trial or to terminate an existing clinical trial;
- adverse regulatory decisions, including failure to receive regulatory approval of our product candidates;
- changes in laws or regulations applicable to our products, including but not limited to clinical trial requirements for approvals;
- adverse developments concerning our CID technology platform and our small molecule drug rimiducid;
- adverse developments concerning our manufacturers;
- our inability to obtain adequate product supply for any approved product or inability to do so at acceptable prices;
- our inability to establish collaborations if needed;
- our failure to commercialize our product candidates;
- additions or departures of key scientific or management personnel;
- unanticipated serious safety concerns related to the use of our product candidates;
- introduction of new products or services offered by us or our competitors;
- announcements of significant acquisitions, strategic partnerships, joint ventures or capital commitments by us or our competitors;
- our ability to effectively manage our growth;
- the size and growth of our initial target markets;
- our ability to successfully treat additional types of diseases and cancers or at different stages;
- actual or anticipated variations in quarterly operating results;
- our cash position;
- our failure to meet the estimates and projections of the investment community or that we may otherwise provide to the public;
- 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;
- changes in the market valuations of similar companies;
- overall performance of the equity markets;
- sales of our common stock by us or our stockholders in the future;
- trading volume of our common stock;
- changes in accounting practices;
- ineffectiveness of our internal controls;

- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;
- significant lawsuits, including patent or stockholder litigation;
- general political and economic conditions; and
- 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. 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 can exert significant control over matters subject to stockholder approval.

As of December 31, 2014, our executive officers, directors, and 5% stockholders beneficially owned approximately 42.3% of our outstanding voting shares. Therefore, these stockholders may have the ability to significantly influence us through this ownership position. These stockholders may be able to significantly influence all matters requiring stockholder approval. For example, these stockholders may be able to significantly influence 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.

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 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 Annual Report 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 completed our initial public 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 our recently completed initial public 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 Annual Report 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.

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

We completed an initial public offering on December 23, 2014. As a new public company, we will incur significant legal, accounting and other expenses that we did not incur as a private company. We are now subject to the reporting requirements of the Exchange Act, which requires, among other things, that we file with 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 an initial public 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, 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 non-routine 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. To remediate our resource weakness, we hired a chief financial officer in November 2014 and in early 2015 we hired a financial reporting manager and contracted with 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. If we are

unable to successfully remediate our material weakness or if we 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.

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 lapse, the trading price of our common stock could decline. As of December 31, 2014, we had outstanding a total of 26,372,592 shares of common stock. Of these shares, only approximately 8,452,500 shares of common stock are freely tradable without restriction in the public market, which number consists of shares of common stock sold in our recently completed initial public 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 our recently completed initial public offering will expire 180 days from the date of our final prospectus, dated December 17, 2014. 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.

The holders of approximately 16.2 million shares of our common stock, or approximately 62% of our total outstanding common stock as of December 31, 2014, are 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. 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.

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.

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

Our management has broad discretion in the application of the net proceeds from our recently completed initial public offering. Because of the number and variability of factors that will determine our use of the net proceeds from our recently completed initial public 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 our common stock. The failure by our management to apply these funds effectively could harm our business. Pending their use, we may invest the net proceeds from our recently completed initial public offering in short-term, investment-grade, interest-bearing securities. These investments may not yield a favorable return to our stockholders. If we do not invest

or apply the net proceeds from our recently completed initial public offering in ways that enhance stockholder value, we may fail to achieve expected financial results, which could cause our stock price to decline.

Anti-takeover provisions under our charter documents and Delaware law could delay or prevent a change of control which could limit the market price of our common stock and may prevent or frustrate attempts by our stockholders to replace or remove our current management.

Our amended and restated certificate of incorporation and amended and restated bylaws contain provisions that could delay or prevent a change of control of our company or changes in our board of directors that our stockholders might consider favorable. Some of these provisions include:

- a board of directors divided into three classes serving staggered three-year terms, such that not all members of the board will be elected at one time;

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- a prohibition on stockholder action through written consent, which requires that all stockholder actions be taken at a meeting of our stockholders;
- a requirement that special meetings of stockholders be called only by the chairman of the board of directors, the chief executive officer, or by a majority of the total number of authorized directors;
- advance notice requirements for stockholder proposals and nominations for election to our board of directors;
- a requirement that no member of our board of directors may be removed from office by our stockholders except for cause and, in addition to any other vote required by law, upon the approval of not less than two-thirds of all outstanding shares of our voting stock then entitled to vote in the election of directors;
- a requirement of approval of not less than two-thirds of all outstanding shares of our voting stock to amend any bylaws by stockholder action or to amend specific provisions of our certificate of incorporation; and
- the authority of the board of directors to issue convertible preferred stock on terms determined by the board of directors without stockholder approval and which convertible preferred stock may include rights superior to the rights of the holders of common stock.

In addition, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporate Law, which may prohibit certain business combinations with stockholders owning 15% or more of our outstanding voting stock. These anti-takeover provisions and other provisions in our amended and restated certificate of incorporation and amended and restated bylaws could make it more difficult for stockholders or potential acquirors to obtain control of our board of directors or initiate actions that are opposed by the then-current board of directors and could also delay or impede a merger, tender offer or proxy contest involving our company. These provisions could also discourage proxy contests and make it more difficult for you and other stockholders to elect directors of your choosing or cause us to take other corporate actions you desire. Any delay or prevention of a change of control transaction or changes in our board of directors could cause the market price of our common stock to decline.

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

The trading market for our common stock will depend in part on the research and reports that securities or industry analysts publish about us or our business. Securities and industry analysts do not currently, and may never, publish research on our company. If no securities or industry analysts commence coverage of our company, the trading price for our stock would likely be negatively impacted. In the event securities or industry analysts initiate coverage, if one or more of the analysts who covers us downgrades our stock or publishes inaccurate or unfavorable research about our business, our stock price may decline. If one or more of these analysts ceases coverage of our company or fails to publish reports on us regularly, demand for our stock could decrease, which might cause our stock price and trading volume to decline.

#### ITEM 1B. Unresolved Staff Comments

Not applicable.

#### ITEM 2. Properties

We lease a 35,250 square foot facility in Houston, Texas for administrative and research and development activities that expires on January 31, 2020, subject to five one-year renewal options. We believe that our existing facilities are adequate to meet our current needs, and that suitable additional alternative spaces will be available in the future on commercially reasonable terms.

#### ITEM 3. Legal Proceedings

From time to time, we may be involved in various claims and legal proceedings relating to claims arising out of our operations. We are not currently a party to any material legal proceedings.

ITEM 4. Mine Safety Disclosures

Not applicable.

## PART II

## ITEM 5. Market for Registrant’s Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities

## Market Information

Our common stock began trading on The NASDAQ Global Market on December 18, 2014 and trades under the symbol “BLCM”. Prior to such time, there was no public market for our common stock.

On March 17, 2015, the last reported sale price of our common stock was \$24.94. The following table sets forth the high and low sales prices per share of our common stock as reported on The NASDAQ Global Market for the period indicated:

	Price Range	
	High	Low
Year Ended December 31, 2014		
Fourth Quarter (commencing December 18, 2014)	\$27.38	\$18.20

## Holders of Record

As of December 31, 2014, there were approximately 110 stockholders of record of our common stock. Certain shares are held in “street” name and accordingly, the number of beneficial owners of such shares is not known or included in the foregoing number.

## Dividend Policy

We have never declared or paid any dividends on our common stock. We currently intend to retain all available funds and any future earnings to support our operations and finance the growth and development of our business and we do not anticipate paying any cash dividends in the foreseeable future. Any future determination related to our dividend policy will be made at the discretion of our board of directors and will depend upon, among other factors, our results of operations, financial condition, capital requirements, contractual restrictions, business prospects and other factors our board of directors may deem relevant. In addition, the terms of our existing line of credit prohibits us from paying dividends.

## Securities Authorized for Issuance Under Equity Compensation Plans

Information about our equity compensation plans is incorporated herein by reference to Item 12 of Part III of this Annual Report.

## Performance Graph

The following stock performance graph illustrates a comparison of the total cumulative stockholder return on our common stock since December 18, 2014, which is the date our common stock first began trading on the NASDAQ Global Market, to two indices: the NASDAQ Biotechnology Index (^NBI) and the NASDAQ Composite Index (^IXIC). The graph assumes an initial investment of \$100 on December 18, 2014 and assumes reinvestment of the full amount of all dividends, if any. The comparisons in the graph are required by the Securities and Exchange Commission and are not intended to forecast or be indicative of possible future performance of our common stock.

The performance graph shall not be deemed to be incorporated by reference by means of any general statement incorporating by reference this Form 10-K into any filing under the Securities Act or the Exchange Act, except to the extent that we specifically incorporate such information by reference, and shall not otherwise be deemed filed under such acts.

## Recent Sales of Unregistered Securities

During the year ended December 31, 2014, we issued and sold the following unregistered securities:

- (1) In January 2014 we entered into our second over-allotment closing of our Series B financing pursuant to amendments to our first Series B stock purchase agreement and second Series B stock purchase agreement. We received proceeds of approximately \$7.3 million for which we issued 1,582,705 shares of Series B convertible preferred stock at a purchase price of \$4.625 per share.
- (2) In the first quarter of 2014, cash consideration of \$200,700 was received and 393,523 shares of common stock were issued upon exercise of common warrants.
- (3) In August 2014 we entered into a Series C convertible preferred stock purchase agreement pursuant to which we received proceeds of approximately \$55 million for which we issued 10,091,743 shares of Series C convertible preferred stock, at a purchase price of \$5.45 per share, and warrants to purchase up to 6,559,598 shares of Series C convertible preferred stock at an exercise price of \$6.00 per share.
- (4) From January 1, 2014 to December 31, 2014, we granted stock options under our 2011 stock option plan to purchase up to an aggregate of 1,047,629 shares of our common stock to our employees, directors and consultants at a weighted-average exercise price of \$7.28 per share. During 2014, holders of 12,615 options, which had been granted in earlier years, exercised their options to purchase shares of our common stock.
- (5) From January 1, 2014 to December 31, 2014, we granted (i) stock options to purchase up to an aggregate of 141,176 shares of our common stock to our employees at a weighted-average exercise price of \$19.00 per share and (ii) a restricted

stock award covering 117,647 shares of our common stock to an employee, each under our 2014 equity incentive plan. None of these options to purchase shares of common stock have been exercised through December 31, 2014.

- (6) In December 2014, in connection with the effectiveness of our registration statement on Form S-1 for our initial public offering, we issued 6,524,195 shares of our Series C convertible preferred stock upon the exercise of outstanding warrants. We received aggregate cash consideration of approximately \$39.1 million for such exercises. The shares of Series C convertible preferred stock issued upon such exercises converted into an aggregate of 3,837,727 shares of our common stock upon the closing of the initial public offering.
- (7) In December 2014, our board of directors declared an accrued dividend of approximately \$3.4 million, payable to the holders of our Series B convertible preferred stock upon the conversion of such shares into shares of our common stock at the closing of the initial public offering. The dividend was payable in cash unless a holder requested that such dividend be paid in shares of our common stock. Thirty-five holders of Series B convertible preferred stock requested that their dividends be paid in shares of our common stock, resulting in the issuance of 168,199 shares of our common stock on December 23, 2014, in lieu of an aggregate of approximately \$3.2 million of cash dividend payments, following the closing of the initial public offering.
- (8) In December 2014, in connection with the closing of our initial public offering, we issued 117,001 shares of our common stock upon the exercise of outstanding warrants. We received aggregate cash consideration of \$49,001 and an aggregate of 641 shares of our common stock issuable under such warrants were forfeited due to net exercises of certain of the warrants.

The offers, sales and issuances of the securities described in paragraphs (1), (2),(3), (6), (7) and (8) were deemed to be exempt from registration under the Securities Act in reliance on Section 4(2) (or Regulation D promulgated thereunder), in that the issuance of securities to the accredited investors did not involve a public offering. The recipients of securities in each of these transactions acquired the securities for investment only and not with a view to or for sale in connection with any distribution thereof and appropriate legends were affixed to the securities issued in these transactions. Each of the recipients of securities in these transactions was an accredited investor under Rule 501 of Regulation D. No underwriters were involved in these transactions.

The offers, sales and issuances of the securities described in paragraphs (4) and (5) were deemed to be exempt from registration under the Securities Act in reliance on Rule 701 in that the transactions were under compensatory benefit plans and contracts relating to compensation as provided under Rule 701. The recipients of such securities were our employees, directors or bona fide consultants and received the securities under our 2006 Stock option Plan, 2011 Stock Option Plan or our 2014 Equity Incentive Plan. Appropriate legends were affixed to the securities issued in these transactions. Each of the recipients of securities in these transactions had adequate access, through employment, business or other relationships, to information about us.

#### Use of Proceeds

On December 17, 2014, we commenced our initial public offering pursuant to a registration statement on Form S-1 (File No. 333- 200328) that was declared effective by the SEC on December 17, 2014 and that registered an aggregate of 7,350,000 shares of our common stock for sale to the public at a price of \$19.00 per share. In addition, at the closing of the initial public offering on December 23, 2014, the underwriters exercised their over-allotment option to purchase 1,102,500 additional shares of our common stock in the initial public offering at the public offering price of \$19.00 per share, for an aggregate offering price of \$160.6 million. The net offering proceeds to us, after deducting underwriting discounts and commissions and offering costs, were approximately \$146.3 million.

The net proceeds from our initial public offering have been invested in highly-liquid money market funds. There has been no material change in the expected use of the net proceeds from our initial public offering as described in our final prospectus filed with the SEC pursuant to Rule 424(b).

## ITEM 6. Selected Financial Data

The following selected financial data should be read in conjunction with our audited financial statements located elsewhere in this Annual Report on Form 10-K and “Item 7. Management’s Discussion and Analysis of Financial Condition and Results of Operations.” Amounts are in thousands, except share and per share data.

We derived the statements of operations data for the years ended December 31, 2014, 2013 and 2012 and the balance sheet data as of December 31, 2014 and 2013 from our audited financial statements included in this annual report. We derived the balance sheet data as of December 31, 2012, from our audited financial statements not included in this report. Our historical results for any prior period are not necessarily indicative of the results to be expected for any future period. You should read the notes to our financial statements for an explanation of the method used to determine the number of shares used in computing basic and diluted net loss per share.

	2014	2013	2012
<b>Statement of Operations:</b>			
Grant revenues	\$1,780	\$1,941	\$1,470
<b>Operating expenses:</b>			
Research and development	11,008	7,050	5,796
ARIAD license restructuring	43,212	—	—
General and administrative	5,398	2,813	1,943
Total operating expenses	59,618	9,863	7,739
Loss from operations	(57,838 )	(7,922 )	(6,269 )
Interest income	35	4	7
Interest expense	(1,791 )	(51 )	(1 )
Change in fair value of warrant liability	(24,371 )	—	—
Net loss	\$(83,965 )	\$(7,969 )	\$(6,263 )
Preferred stock dividends	(1,432 )	(1,093 )	(757 )
Net loss attributable to common stockholders	\$(85,397 )	\$(9,062 )	\$(7,020 )
Basic and diluted net loss per share	\$(34.04 )	\$(5.25 )	\$(4.26 )
Weighted average common shares outstanding— basic and diluted	2,508,960	1,795,992	1,648,198

	2014	2013	2012
<b>Balance Sheet Data:</b>			
Cash and cash equivalents	\$191,602	\$11,168	\$1,632
Working capital	189,586	9,963	256
Total assets	195,794	14,942	5,186
Convertible preferred stock	—	39,926	21,658
Accumulated deficit	(112,944)	(28,979)	(21,010)
Total stockholders’ equity (deficit)	191,636	(28,152)	(19,473)

## ITEM 7. Management's Discussion and Analysis of Financial Condition and Results of Operations

You should read the following discussion and analysis together with "Item 6. Selected Financial Data" and our financial statements and related notes included elsewhere in this Annual Report. The following discussion contains forward-looking statements that involve risks and uncertainties. Our actual results could differ materially from those expressed or implied in any forward-looking statements as a result of various factors, including those set forth under the caption "Item 1A. Risk Factors."

### 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. 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.

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. 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.

- 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. 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. 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 fourth quarter of 2015.
- 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.

In addition, our preclinical product candidates are designed to overcome the current limitations of CAR-T and TCR therapies and include the following:

- BPX-401. We are developing a CIDECAR product candidate, BPX-401, as a next-generation CAR T cell therapy for hematological cancers that express the CD19 antigen.
- 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.
- 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.

We expect to file INDs for BPX-701 in the fourth quarter 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 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.

## Recent Developments

In December 2014, we completed our initial public offering of shares of our common stock which resulted in aggregate gross proceeds to us of approximately \$160.6 million and net offering proceeds to us, after deducting underwriting discounts and commissions and offering costs, of approximately \$146.3 million. For additional information about our initial public offering see Note 9 to the financial statements included herein, and see ‘Liquidity and Capital Resources’, below.

On December 23, 2014, the closing of our initial public offering triggered an acceleration of the payment of \$15 million due to ARIAD under our omnibus amendment agreement with ARIAD and our promissory note issued to ARIAD. As a result of such acceleration, on December 29, 2014, we paid to ARIAD an aggregate amount of \$35 million, which included an additional payment of



\$20 million to extinguish the promissory note. Pursuant to the terms of the omnibus amendment agreement, ARIAD returned to us all of the 677,463 shares of our common stock then held by ARIAD. For additional information, see Note 12 to the financial statements included herein.

## Financial Operations Overview

### Revenue

To date, we have only recognized revenue from government grants and we have not generated any product revenue. We have received funds from the Cancer Prevention and Research Institute of Texas, or CPRIT, and the National Institute of Health, or NIH, which are awarded based on the progress of the program being funded. In cases when the grant money is not received until expenses for the program are incurred, we accrue the revenue based on the costs incurred for the programs associated with the grant.

During 2011, we entered into a grant agreement with CPRIT for approximately \$5.7 million covering a three year period from July 1, 2011 through June 30, 2014. The grant initially allowed us to receive funds in advance of costs and allowable expenses being incurred. On a quarterly basis, we are required to submit a financial reporting package outlining the nature and extent of reimbursed costs under the grant. At the end of each period, any excess funds received in advance, or paid prior to reimbursement, result in a deferred liability or grant receivable. The CPRIT grant has expired as of June 30, 2014. We recorded a grant receivable from CPRIT of \$0.3 million at December 31, 2014, which we collected during the first quarter of 2015.

During 2013, the Company entered into a grant agreement with the National Institute of Health (NIH). The grant is a modular five year grant with funds being awarded each year based on the progress of the program being funded. Grant money is not received until expenses for the program are incurred. We have been awarded approximately \$0.7 million to date, of which \$0.5 million has been received. We accrue the revenue based on the costs incurred for the programs associated with the grant.

In the future, we may generate revenue from a combination of product sales, government or other third-party grants, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements or a combination of these approaches. We expect that any revenue we generate will fluctuate from quarter to quarter as a result of the timing and amount of license fees, milestone and other payments, and the amount and timing of payments that we receive upon the sale of our products, to the extent any are successfully commercialized. If we fail to complete the development of our product candidates in a timely manner or obtain regulatory approval of them, our ability to generate future revenue, and our results of operations and financial position, would be materially adversely affected.

### Research and Development Expenses

To date, our research and development expenses have related primarily to the development of our CID platform and the identification and development of our product candidates. Research and development expenses consist of expenses incurred in performing research and development activities, including compensation and benefits for research and development employees and consultants, facilities expenses, overhead expenses, cost of laboratory supplies, manufacturing expenses, fees paid to third parties and other outside expenses.

Research and development costs are expensed as incurred. Clinical trial and other development costs incurred by third parties are expensed as the contracted work is performed. We accrue for costs incurred as the services are being provided by monitoring the status of the clinical trial or project and the invoices received from our external service providers. We adjust our accrual as actual costs become known. Where contingent milestone payments are due to third parties under research and development arrangements, the milestone payment obligations are expensed when the milestone events are achieved.

We utilize our research and development personnel and infrastructure resources across several programs, and many of our costs are not specifically attributable to a single program. Accordingly, we cannot state precisely our total costs incurred on a program-by-program basis.

Research and development activities are central to our business model. Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. We expect our research and development expenses to increase over the next several years as we seek to conduct our ongoing and planned clinical trials for BPX-501, BPX-201, BPX-401, BPX-601 and BPX-701 and as we selectively develop additional product candidates. However, it is difficult to determine with certainty the duration and completion costs of our current or future preclinical programs and clinical trials of our product candidates.

The duration, costs and timing of clinical trials and development of our product candidates will depend on a variety of factors that include, but are not limited to, the following:

- per patient clinical trial costs;
- the number of patients that participate in the clinical trials;
- the number of sites included in the clinical trials;
- the process of collection, differentiation, selection and expansion of immune cells for our cellular immuno-therapies;
- the countries in which the clinical trials are conducted;
- the length of time required to enroll eligible patients;
- the number of doses that patients receive;
- the drop-out or discontinuation rates of patients;
- potential additional safety monitoring or other studies requested by regulatory agencies;
- the duration of patient follow-up; and
- the efficacy and safety profile of the product candidates.

The following table indicates our research and development expense by project/category for the periods indicated:

	YEARS ENDED			
	DECEMBER 31,			
				Total Program  Inception Through  December 31, 2014
Program	2014	2013	2012	
BPX-101	\$—	\$—	\$—	\$6,478,453
BPX-201	2,055,805	1,563,324	1,943,433	6,636,146
BPX-501	6,041,376	3,061,500	2,239,482	12,029,184
General	2,911,166	2,424,596	1,613,318	8,314,822
<b>Total</b>	<b>\$11,008,347</b>	<b>\$7,049,420</b>	<b>\$5,796,233</b>	<b>\$33,458,605</b>

In addition, the probability of success for each product candidate will depend on numerous factors, including competition, manufacturing capability and commercial viability. We will determine which programs to pursue and how much to fund each program in response to the scientific and clinical success of each product candidate, as well as an assessment of each product candidate's commercial potential

#### General and Administrative Expenses

General and administrative expenses consist primarily of salaries and other related costs, including share-based compensation, for personnel in executive, finance, accounting, business development, legal and human resources functions. Other significant costs include facility costs not otherwise included in research and development expenses, legal fees relating to patent and corporate matters, insurance costs and professional fees for consultancy, legal, accounting, audit and investor relations.

We anticipate that our general and administrative expenses will increase in the future to support our continued research and development activities, potential commercialization of our product candidates and the increased costs of operating as a public company. These increases will likely include increased costs related to the hiring of additional personnel and fees to outside consultants, lawyers and accountants, among other expenses. Additionally, we anticipate

increased costs associated with being a public company, including expenses related to services associated with maintaining compliance with NASDAQ listing rules and SEC requirements, insurance and investor relations costs.

Other Income (Expense)

Other income (expense), net consists of interest income, interest expense and the change in the fair value of a warrant liability.

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## Results of Operations

## Comparison of the Years Ended December 31, 2014 and 2013

The following table sets forth our results of operations for the years ended December 31, 2014 and 2013:

	YEARS ENDED		
	DECEMBER 31, 2014	2013	CHANGE \$
(in thousands)			
Grant revenues	\$1,780	\$1,941	\$(161 )
Operating expenses:			
Research and development	11,008	7,050	3,958
ARIAD license restructuring	43,212	—	43,212
General and administrative	5,398	2,813	2,585
Total operating expenses	59,618	9,863	49,755
Loss from operations	(57,838)	(7,922)	(49,916 )
Other income (expense):			
Interest income	35	4	31
Interest expense	(1,791 )	(51 )	(1,740 )
Change in fair value of warrant liability	(24,371)	—	(24,371 )
Total other income (expense)	(26,127)	(47 )	(26,080 )
Net loss	\$(83,965)	\$(7,969)	\$(75,996 )

## Grant Revenues

Grant revenues were \$1.8 million and \$1.9 million for the years ended December 31, 2014 and 2013, respectively. The decrease in grant revenues is primarily due to the expiration of the CPRIT grant in June 2014.

## Research and Development Expenses

Research and development expenses were \$11.0 million and \$7.1 million for the years ended December 31, 2014 and 2013, respectively. The increase in research and development expenses is primarily due to an increase in manufacturing of \$2.0 million and clinical expenses of \$0.8 million as a result of increased patient enrollment in our clinical trials for BPX-501 and BPX-201.

## ARIAD License Restructuring

On October 3, 2014, the Company entered into an omnibus amendment agreement with ARIAD, under which the Company agreed to make payments of \$50.0 million in exchange for an expansion of the license field, the termination of all obligations to make milestone and royalty payments to ARIAD in the future and the return of 677,463 shares of Bellicum common stock that ARIAD held.

In connection with the amendment, the Company made an initial payment of \$15.0 million and issued a promissory note to ARIAD for a principal amount of \$35.0 million. Per the promissory note terms, the principal would not accrue interest unless the Company was in default, in which case it would accrue at a rate of 10% per annum. In December 2014 following the Company's IPO, the Company paid the remaining \$35.0 million and ARIAD returned all 677,463 shares of common stock of the Company that ARIAD held. The license transaction was valued on the date of the transaction and the note was discounted to fair market value at a 10% rate. This resulted in license expense of \$43.2

million, repurchase of the Company's common stock for \$5.1 million, and interest expense of \$1.7 million. The Company has recorded the returned shares of common stock as treasury stock. See Note 12 to the financial statements included herein.

#### General and Administrative Expenses

General and administrative expenses were \$5.4 million and \$2.8 million for the years ended December 31, 2014 and 2013, respectively. The increase in general and administrative expenses was due to our overall growth, including an increase in personnel, legal and accounting expenses, costs related to facilities, travel and entertainment expenses and depreciation expense related to equipment.

## Other Income (Expense)

Other expense was \$26.1 million and \$47,000 for the years ended December 31, 2014 and 2013, respectively. The increase in other expense is primarily due to the change in value of the warrant liability of \$24.4 million and imputed interest expense from the ARIAD license restructuring of \$1.7 million. In connection with the August 2014 issuance of Series C convertible preferred stock, Bellicum issued warrants to purchase 6,559,598 shares of Series C convertible preferred stock with an exercise price of \$6.00 per share which were convertible into 3,858,549 common shares. The fair value of the warrants on the date of issuance of \$9.4 million, as determined using the Black-Scholes option-pricing model, was recorded as a warrant liability. The Series C warrants were revalued both at September 30, 2014 to \$10.6 million, and again revalued at the time of exercise in December 2014 to \$33.8 million. The increase in the calculated fair value from the issuance date to the remeasurement dates resulted in non-cash expense of \$24.4 million in 2014, of which \$23.2 million was a fourth quarter expense. As all the warrants were either exercised or expired in December 2014, there will be no future charges in connection with the warrants.

## Comparison of the Years Ended December 31, 2013 and 2012

The following table sets forth our results of operations for the years ended December 31, 2013 and 2012:

	YEAR ENDED		
	DECEMBER 31,		CHANGE
	2013	2012	\$
(in thousands)			
Grant revenues	\$1,941	\$1,470	\$ 471
Operating expenses:			
Research and development	7,050	5,796	1,254
General and administrative	2,813	1,943	870
Total operating expenses	9,863	7,739	2,124
Loss from operations	(7,922)	(6,269)	(1,653 )
Other income (expense):			
Interest income	4	7	(3 )
Interest expense	(51 )	(1 )	(50 )
Total other income (expense)	(47 )	6	(53 )
Net loss	\$(7,969)	\$(6,263)	\$ (1,706 )

## Grant Revenues

Grant revenues were \$1.9 million and \$1.5 million for the years ended December 31, 2013 and 2012, respectively. The increase in grant revenues was due to the addition of the grant from the NIH, received in April 2013.

## Research and Development Expenses

Research and development expenses were \$7.1 million and \$5.8 million for the years ended December 31, 2013 and 2012, respectively. The increase in research and development expenses was primarily due to the increase in personnel and clinical expenses as a result of increased patient enrollment in the clinical trials of BPX-501 and BPX-201, as well as an increase in total patient costs of \$0.5 million, which included \$0.2 million of clinical site costs and \$0.2 million of specific patient treatment costs. BPX-501 clinical and manufacturing costs increased by \$0.3 million and \$0.4 million, respectively, for the year ended December 31, 2013 when compared to the previous year. BPX-201 clinical costs increased by \$0.2 million and manufacturing costs decreased by \$0.6 million, for the year ended December 31, 2013 when compared to the previous year.

### General and Administrative Expenses

General and administrative expenses were \$2.8 million and \$1.9 million for the years ended December 31, 2013 and 2012, respectively. The increase in expenses was due to our overall growth, including an increase in personnel, legal and accounting expenses, costs related to facilities, travel and entertainment expenses, and depreciation expense related to equipment.

### Other Income (Expense)

Other expense was \$47,000 and other income was \$6,000 for the years ended December 31, 2013 and 2012, respectively. The change was primarily due to additional interest expense that was incurred due to the increase in the outstanding principal balance under the line of credit described below.



## Liquidity and Capital Resources

### Sources of Liquidity

We are a clinical stage biopharmaceutical company with a limited operating history. To date, we have financed our operations primarily through equity and debt financings and receipt of grants to fund our research and development programs. We have not generated any revenue from the sale of any products. As of December 31, 2014 and 2013, we had available cash and cash equivalents of \$191.6 million and \$11.2 million, respectively. Our cash and cash equivalents are held in cash and money market accounts. Cash in excess of immediate requirements is invested with a view toward liquidity and capital preservation, and we seek to minimize the potential effects of concentration and degrees of risk.

We were party to a line of credit which was executed in 2012 for \$1.0 million. The annual interest rate was equal to the prime rate plus 2.75%. We took advances under this line of credit to fund equipment purchases and other capital expenditures. During 2013, we were advanced \$0.6 million under the line of credit. Principal payments on the \$1.0 million line of credit began in July 2013 and were to be paid over 30 months. During 2014, the line of credit was amended to include a credit extension up to \$0.5 million. Interest accrued at a rate of prime plus 2.75% from the date of each advance. Any advances that were outstanding on the credit extension were payable in 24 equal monthly installments of principal, plus all accrued interest, beginning on April 1, 2015. During the year ended December 31, 2014, \$0.4 million was advanced under the credit extension and \$1.2 million of principal and interest payments were made. The line of credit was paid off as of December 31, 2014.

On February 12, 2013, we received \$3.5 million of cash proceeds through the issuance of promissory notes, bearing interest at 0.21% per annum from February 12, 2013 through July 31, 2013. On July 31, 2013, in connection with the issuance of Series B convertible preferred stock, we repaid the notes with 757,497 shares of Series B convertible preferred stock at a conversion price of \$4.625 per share. The converted balances consisted of \$3.5 million of principal and \$3,426 of outstanding interest payable.

In the first quarter of 2014, we issued 1,582,705 shares of our Series B convertible preferred stock for net proceeds of \$7.3 million, and received \$0.2 million pursuant to the exercise of common warrants.

In August 2014, we completed a private placement of 10,091,743 shares of Series C convertible preferred stock and warrants to purchase up to 6,559,598 shares of Series C convertible preferred stock and received gross proceeds of \$55.0 million, resulting in net proceeds of \$51.5 million. The warrants had an exercise price of \$6.00 per share. The warrants provided for automatic termination upon the date immediately following the date of effectiveness of our registration statement on Form S-1 in connection with our initial public offering. As a result, substantially all of such warrants were exercised in December 2014. The Company received gross proceeds of approximately \$39.1 million from the exercise of warrants, resulting in net proceeds of \$38.4 million. See Note 9 to the financial statements included herein.

In December 2014, we completed our initial public offering of shares of our common stock which resulted in aggregate gross proceeds to us of approximately \$160.6 million and net offering proceeds to us, after deducting underwriting discounts and commissions and offering costs, of approximately \$146.3 million.

In conjunction with the initial public offering, substantially all of the common warrants were exercised resulting in additional proceeds of \$49,001. Also in conjunction with the initial public offering, \$3.4 million of accrued Series B dividends were paid, of which \$0.2 million was paid in cash and the remainder was paid by issuance of 168,199 shares of common stock.

### Funding Requirements

Our primary uses of capital are, and we expect will continue to be, compensation and related expenses, third-party clinical research and development services, laboratory and related supplies, clinical costs, legal and other regulatory expenses, facility costs and general overhead costs. Specifically, we expect to use capital to expand our manufacturing capabilities.

The successful development of any product candidate is highly uncertain. As such, at this time, we cannot reasonably estimate or know the nature, timing and costs of the efforts that will be necessary to complete the development of BPX-501 or our other current and future product candidates. We are also unable to predict when, if ever, material net cash inflows will commence from the sale of product candidates. This is due to the numerous risks and uncertainties associated with developing medical treatments, including the uncertainty of:

- successful enrollment in, and completion of, clinical trials;
- receipt of marketing approvals from applicable regulatory authorities;
- making arrangements with third-party manufacturers;

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- obtaining and maintaining patent and trade secret protection and regulatory exclusivity; and
- launching commercial sales of our products, if and when approved, whether alone or in collaboration with others.

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

Because all of our product candidates are in the early stages of clinical and preclinical development and the outcome of these efforts is uncertain, we cannot estimate the actual amounts necessary to successfully complete the development and commercialization of product candidates or whether, or when, we may achieve profitability. Until such time, if ever, as we can generate substantial product revenue, we expect to finance our cash needs through a combination of equity or debt financings and collaboration arrangements.

We plan to continue to fund our operations and capital funding needs through equity and/or debt financing. We may also consider new collaborations or selectively partnering our technology. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interests of our stockholders will be diluted, and the terms may include liquidation or other preferences that adversely affect the rights of our existing stockholders. 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. Any of these actions could harm our business, results of operations and future prospects.

#### Outlook

Based on our research and development plans and our timing expectations related to the progress of our programs, we expect that our cash and cash equivalents as of December 31, 2014, which included the net proceeds from our recently completed initial public offering, will enable us to fund our operating expenses and capital expenditure requirements through at least the first half of 2017. We have based this estimate on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we currently expect. Furthermore, our operating plan may change, and we may need additional funds to meet operational needs and capital requirements for product development and commercialization sooner than planned. Because of the numerous risks and uncertainties associated with the development and commercialization of our product candidates and the extent to which we may enter into additional collaborations with third parties to participate in their development and commercialization, we are unable to estimate the amounts of increased capital outlays and operating expenditures associated with our current and anticipated clinical trials. Our future funding requirements will depend on many factors, as we:

- initiate or continue clinical trials of BPX-501 and any other product candidates;
- continue the research and development of our product candidates;
- seek to discover additional product candidates;
- seek regulatory approvals for our product candidates that successfully complete clinical trials;
- establish a sales, marketing and distribution infrastructure and scale-up manufacturing capabilities to commercialize products which receive regulatory approval;
- enhance operational, financial and information management systems and hire additional personnel, including personnel to support development of our product candidates and, if a product candidate is approved, our commercialization efforts; and
- incur additional costs associated with becoming a public company.

#### Cash Flows

The following table sets forth a summary of our cash flows for the years ended December 31, 2014, 2013 and 2012:

	FOR THE YEARS ENDED		
	DECEMBER 31		
	2014	2013	2012
(in thousands)			
Net cash used in operating activities	\$(57,308 )	\$(7,613 )	\$(7,744)
Net cash used in investing activities	(804 )	(366 )	(2,047)
Net cash provided by financing activities	238,546	17,515	3,516
Net cash inflow (outflow)	\$180,434	\$9,536	\$(6,275)

### Operating Activities

Net cash used in operating activities of \$57.3 million for the year ended December 31, 2014, was comprised of a net loss of \$84.0 million, which included depreciation expense of \$0.7 million, share-based compensation expense of \$0.9 million and a \$24.4 million non-cash charge for the revaluation of the Series C Warrants. Net cash used in operating activities was also comprised of the following primary components: a decrease in grant receivables of \$0.4 million, an increase in prepaid expenses and other current assets of \$1.1 million, a decrease in other assets of \$0.3 million, an increase in accounts payable of \$0.7 million, an increase in accrued payroll of \$0.3 million, and an increase in deferred manufacturing costs of \$0.2 million.

Net cash used in operating activities was \$7.6 million for the year ended December 31, 2013, which was derived from a net loss of \$8.0 million, in addition to the following primary components: a decrease in prepaid expenses and other assets of \$0.3 million, an increase in accounts payable, accrued payroll, and accrued liabilities of \$0.7 million, a decrease in deferred revenue-grants of \$1.0 million, and share-based compensation of \$0.4 million.

Net cash used in operating activities was \$7.7 million for the year ended December 31, 2012, which was derived from a net loss of \$6.3 million, in addition to the following primary components: a decrease in prepaid expenses and other assets of \$0.8 million, an increase in accounts payable and accrued payroll of \$0.4 million, a decrease in deferred revenue of \$1.5 million, and share based compensation of \$0.1 million.

### Investing Activities

Net cash used in investing activities for the year ended December 31, 2014 was \$0.8 million, which was derived solely from the purchase of property and equipment.

Net cash used in investing activities for the year ended December 31, 2013 was \$0.4 million, which was derived solely from the purchase of property and equipment.

Net cash used in investing activities for the year ended December 31, 2012 was \$2.0 million, which was derived solely from the purchase of property and equipment.

### Financing Activities

Net cash provided by financing activities for the year ended December 31, 2014 was \$238.5 million, which was derived from approximately \$146.3 million in net proceeds from our recent initial public offering, \$101.5 million from the issuance of convertible preferred stock and the exercise of warrants, offset by \$3.5 million of issuance costs, proceeds from the exercise of common warrants other than in our initial public offering of \$0.3 million, payment of \$5.1 million for repurchase of stock held by ARIAD, payments totaling \$0.2 million for series B dividends, and proceeds from the line of credit of \$0.4 million, which were offset by payments on the line of credit of \$1.2 million. See Note 9 to the audited Financial Statements included herein.

Net cash provided by financing activities for the year ended December 31, 2013 was \$17.5 million, which was derived from proceeds from issuance of preferred stock of \$13.7 million, proceeds from notes payable of \$3.5 million and proceeds from the line of credit of \$0.6 million, offset by payments on the line of credit of \$0.2 million.

Net cash provided by financing activities for the year ended December 31, 2012 was \$3.5 million, which was derived from proceeds of \$0.5 million from the line of credit, and \$3.1 million from issuance of Series B Preferred Stock, offset by \$0.1 million of issuance costs. See Note 9 to the audited Financial Statements included herein.

### Contractual Obligations

Our contractual obligations as of December 31, 2014 were as follows:

		LESS THAN	1 TO	3 TO	MORE THAN
	TOTAL	1 YEAR	3 YEARS	5 YEARS	5 YEARS
Operating lease agreements (1)	\$48,000	\$48,000	\$—	\$—	\$ —
Contract manufacturing arrangements (2)	914,463	656,608	257,855	—	—
Facility lease agreement (3)	5,379,584	980,625	2,116,481	2,189,210	93,268
License agreements (4)	705,000	57,500	480,000	45,000	122,500
Total contractual obligations	\$7,047,047	\$1,742,733	\$2,854,336	\$2,234,210	\$ 215,768

(1) In March 2013 we entered into a two-year manufacturing facility agreement for cell processing for a clinical trial. In February 2015, the agreement was extended for an additional two years.

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- (2) In December 2011 we entered into a service agreement with a third party to perform manufacturing processes for our products. Under the terms of the agreement we agreed to purchase a minimum of 110 batches of product at a cost of \$3,400 per batch plus materials. See Note 8 to the financial statements included herein. In December 2013 we entered into a service agreement to perform manufacturing processes for our products in Europe. The agreement expires in October 2016.
- (3) In December 2012, we entered into a five-year office lease agreement. During 2013, the lease was amended to include additional space. During 2014, the lease was amended again to include additional space and extend the term of the lease. After the 2013 and 2014 amendments, the leased premises totals 35,250 square feet. The lease includes escalating base rent payments, which initially increased on November 1, 2013, and then increased again on December 1, 2013. An additional base rent increase will become effective February 1, 2015 as a result of the 2014 lease modification. Subsequently, an increase in the base rent payment will occur during the first month of each year, and remain constant for the next 11 months. During the last month of each year, the monthly base rent payment will increase yet again. This escalating base rent payment structure will continue through the expiration of the lease on January 31, 2020. See Note 13 to the financial statements included herein.
- (4) We have entered into several license agreements under which we obtained rights to certain intellectual property. Under the agreements, we could be obligated for payments upon successful completion of clinical and regulatory milestones regarding the products covered by this license. The obligations listed in the table above represent estimates of when the milestones will be achieved. We cannot assure that the timing of the milestones will be completed when estimated or at all.

#### Critical Accounting Policies and Significant Estimates

Our financial statements are prepared in accordance with U.S. generally accepted accounting principles, or GAAP. The preparation of these financial statements requires us to make estimates and assumptions that affect the reported amounts of assets, liabilities, revenues, costs and expenses and related disclosures. We base our estimates on historical experience and on various other assumptions that we believe to be reasonable under the circumstances. In many instances, we could have reasonably used different accounting estimates, and in other instances changes in the accounting estimates are reasonably likely to occur from period to period. Accordingly, actual results could differ significantly from management's estimates. To the extent that there are material differences between these estimates and actual results, our future financial statement presentation, financial condition, results of operations and cash flows will be affected. While our significant accounting policies are described in the Notes to our financial statements, we believe that the accounting policies discussed below are critical to understanding our historical and future performance, as these policies related to the more significant areas involving management's judgments and estimates.

**Revenue Recognition**—We have not yet generated any revenue from product sales. Our sole source of revenue is grant revenue related to a \$5.7 million research grant received from CPRIT, covering a three-year period from July 1, 2011 through June 30, 2014 and a \$0.7 million research grant from NIH. Grant payments received prior to our performance of work required by the terms of the research grant are recorded as deferred revenue and recognized as grant revenue once work is performed and qualifying costs are incurred.

**Licenses and Patents**—Licenses and patent costs are expensed as incurred. Costs related to the license of patents from third parties and internally developed patents are classified as research and development expenses. Legal costs related to patent applications and maintenance are classified as general and administrative expenses.

**Research and Development**—Research and development expenses include salaries of research and development personnel and related payroll expenses, consulting fees, laboratory costs, manufacturing costs, and clinical trial expenses. All costs for research and development are expensed as incurred.

**Contract Manufacturing Services**—Contract manufacturing services are expensed as incurred. Prepaid costs are capitalized and amortized as services are performed.

Share-Based Compensation—Share-based compensation cost is measured at the date of grant based on the estimated fair value of the award, net of estimated forfeitures. We estimate the grant date fair value and the resulting share-based compensation expense using the Black-Scholes option pricing model. The grant date fair value of a share-based award is recognized as an expense over the requisite service period of the award on a straight-line basis.

We account for share-based compensation arrangements with non-employees using a fair value approach. The fair value of these options is measured using the Black-Scholes option pricing model reflecting an expected life that is assumed to be the remaining contractual life of the option. The compensation costs of these arrangements are subject to re-measurement over the vesting terms as earned.



We determine the fair value of each grant of stock options using the estimated fair value of our common stock (prior to the IPO) and the assumptions set forth below. Each of these inputs is subjective and generally requires significant judgment.

Our board of directors intends all options granted to be exercisable at a price per share not less than the per share fair value of our common stock underlying those options on the date of grant. The estimated fair value of our common stock prior to the IPO was determined at each valuation date in accordance with the guidelines outlined in the American Institute of Certified Public Accountants Practice Aid, Valuation of Privately-Held-Company Equity Securities Issued as Compensation. Our board of directors, with the assistance of management, developed these valuations using significant judgment and taking into account numerous factors, including Company developments, market conditions and independent third-party valuations as of December 31, 2011, 2012 and 2013, July 31, 2014, and October 15, 2014.

For all option grant dates through December 31, 2014 (other than the option grants to Alan A. Musso, C.P.A., C.M.A. and Jon P. Stonehouse which became effective on December 17, 2014 and were priced at the price at which we issued shares of our common stock to the public in our IPO), the enterprise value was determined based on a Probability Weighted Expected Return Method, or PWERM, Option Pricing Method, or the OPM backsolve method. The allocation of these enterprise values to each part of our capital structure, including our common stock, was done based on OPM. OPM treats the rights of the holders of preferred and common shares as equivalent to call options on any value of the enterprise above certain break points of value based upon the liquidation preferences of the holders of preferred shares, as well as their rights to participation and conversion. Thus, the estimated value of the common stock can be determined by estimating the value of its portion of each of these call option rights. The OPM backsolve method derives the implied equity value of a company from a recent transaction involving the company's own securities issued on an arms-length basis. The Discounted Cash Flow method estimates value based on the expectation of future net cash flows, which are then discounted back to the present using a rate of return derived from alternative companies of similar type and risk profile. Under the PWERM the value is estimated based upon analysis of future values for the enterprise under varying scenarios, probabilities are ascribed to these scenarios based on expected future outcomes.

As of the closing of our recently completed initial public offering, the fair value of our common stock was determined based on the closing price of our common stock on the NASDAQ Global Market.

**Income Taxes**—Income taxes are accounted for under the asset and liability method. Deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax bases. This method also requires the recognition of future tax benefits such as net operating loss and tax credit carry forwards, to the extent that realization of such benefits is more likely than not. A valuation allowance is recorded when the realization of future tax benefits is uncertain. We record a valuation allowance for the full amount of deferred tax assets, which would otherwise be recorded for tax benefits relating to the operating loss and tax credit carryforwards, as realization of such deferred tax assets cannot be determined to be more likely than not.

Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled. The effect on deferred tax assets and liabilities of a change in tax rates is recognized in the statement of operations in the period that includes the enactment date.

We account for uncertain tax positions in accordance with the provisions of the Accounting Standards Codification (ASC) 740, Income Taxes. When uncertain tax positions exist, we recognize the tax benefit of tax positions to the extent that the benefit will more likely than not be realized. The determination as to whether the tax benefit will more likely than not be realized is based upon the technical merits of the tax position as well as consideration of the available facts and circumstances. As of December 31, 2014 and 2013, we had no uncertain tax positions and no

interest or penalties have been charged to us for the years ended December 31, 2014 and 2013. If incurred, we will classify any interest and penalties as a component of interest expense and operating expense, respectively. We are subject to routine audits by taxing jurisdictions; however, there are currently no audits for any tax periods in progress. The tax years 2004 through 2014 remain open to examination by the U.S. Internal Revenue Service.

#### Recently Issued Accounting Pronouncements

In June 2014, the FASB issued Accounting Standards Update No. 2014-12 Compensation – Stock Compensation (ASU No. 2014-12), which clarified the accounting for share-based payments when the terms of an awards provide that a performance target could be achieved after the requisite service period. ASU No. 2014-12 is effective for annual reporting purposes beginning after December 15, 2015, with early adoption allowed. The Company does not believe ASU No. 2014-12 will have a material effect on its financial statements.

In August 2014, the FASB issued Accounting Standard Update No. 2014-15, Presentation of Financial Statements - Going Concern (ASU No. 2014-15, Subtopic 205-40), which requires management to assess an entity's ability to continue as a going concern by incorporating and expanding upon certain principles that are currently in U.S. auditing standards. Specifically, ASU 2014-15 provides

a definition of the term substantial doubt and requires an assessment for a period of one year after the date that the financial statements are issued (or available to be issued). It also requires certain disclosures when substantial doubt is alleviated as a result of consideration of management's plans and requires an express statement and other disclosures when substantial doubt is not alleviated. ASU No. 2014-15 is effective for fiscal years, and interim periods within those years, beginning after December 15, 2016, and early application is permitted.

The Company has evaluated other recent accounting pronouncements and believes that none of them will have a material effect on the Company's financial statements.

#### Off-Balance Sheet Arrangements

We do not have any off-balance sheet arrangements (as defined by applicable regulations of the SEC) that are reasonably likely to have a current or future material effect on our financial condition, results of operations, liquidity, capital expenditures or capital resources.

#### JOBS Act

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

#### ITEM 7A. Quantitative and Qualitative Disclosures About Market Risk

The primary objective of our investment activities is to preserve our capital to fund our operations. We also seek to maximize income from our investments without assuming significant risk. To achieve our objectives, we maintain a portfolio of cash equivalents and investments in a variety of securities of high credit quality. As of December 31, 2014, we had cash and cash equivalents of \$191.6 million consisting of cash and money market accounts in highly rated financial institutions in the United States.

A portion of our investments may be subject to interest rate risk and could fall in value if market interest rates increase. However, because our investments are primarily short-term in duration, we believe that our exposure to interest rate risk is not significant and a 1% movement in market interest rates would not have a significant impact on the total value of our portfolio. We actively monitor changes in interest rates.

#### ITEM 8. Financial Statements and Supplementary Data

##### Index to Financial Statements

The financial statements of Bellicum Pharmaceuticals, Inc. listed below are set forth in Item 8 of this Annual Report for the year ended December 31, 2014:

<u>Report of Independent Registered Public Accounting Firm</u>	Page
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<u>Statements of Operations</u>	73
<u>Statements of Redeemable and Convertible Preferred Stock and Stockholders' Equity (Deficit)</u>	74
<u>Statements of Cash Flows</u>	75
<u>Notes to the Financial Statements</u>	76

Report of Independent Registered Public Accounting Firm

The Board of Directors of Bellicum Pharmaceuticals, Inc.

We have audited the accompanying balance sheets of Bellicum Pharmaceuticals, Inc. as of December 31, 2014 and 2013, and the related statements of operations, redeemable and convertible preferred stock and stockholders' equity (deficit) and cash flows for each of the three years in the period ended December 31, 2014. These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audits.

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

In our opinion, the financial statements referred to above present fairly, in all material respects, the financial position of Bellicum Pharmaceuticals, Inc. at December 31, 2014 and 2013, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2014, in conformity with U.S. generally accepted accounting principles.

/s/ Ernst & Young LLP

Houston, Texas

March 20, 2015

## Bellicum Pharmaceuticals, Inc.

## Balance Sheets

December 31, 2014    December 31, 2013

ASSETS	December 31, 2014	December 31, 2013
<b>ASSETS</b>		
Current assets:		
Cash and cash equivalents	\$ 191,601,611	\$ 11,167,585
Accounts receivable – grants	297,903	745,541
Prepaid expenses and other current assets	1,322,268	254,068
Total current assets	193,221,782	12,167,194
Property and equipment, net of accumulated depreciation	2,427,435	2,289,919
Other assets	145,023	484,525
<b>TOTAL ASSETS</b>	<b>\$ 195,794,240</b>	<b>\$ 14,941,638</b>
<b>LIABILITIES AND STOCKHOLDERS' EQUITY (DEFICIT)</b>		
Current liabilities:		
Accounts payable	\$ 1,209,006	\$ 549,594
Accrued expenses	1,431,425	664,173
Accrued payroll	730,918	470,961
Current portion of line of credit	—	400,000
Deferred revenue	13,122	—
Current portion of deferred rent	97,336	102,713
Current portion of deferred manufacturing costs	154,286	17,000
Total current liabilities	3,636,093	2,204,441
Long-term liabilities:		
Line of credit	—	400,000
Deferred rent	209,531	288,941
Deferred manufacturing costs	312,800	274,267
<b>TOTAL LIABILITIES</b>	<b>4,158,424</b>	<b>3,167,649</b>
Commitments and contingencies:		
Preferred stock whose redemption is outside the control of the issuer:		
Series A convertible, redeemable preferred stock,		
\$0.01 par value; 2,800,000 shares authorized;		
2,544,539 shares issued and outstanding as of		
December 31, 2013; redemption value of \$7,633,617		
at December 31, 2013.	—	7,633,617
Series B convertible, redeemable preferred stock,		
\$0.01 par value; 8,900,000 shares authorized;		
6,563,283 shares issued and outstanding at		
December 31, 2013; redemption value of \$32,292,269		
at December 31, 2013.	—	32,292,269

Stockholders' Equity (Deficit):

Common stock: \$0.01 par value; at December 31, 2014

200,000,000 shares authorized; 27,050,055 shares issued

and 26,372,592 outstanding. At December 31, 2013

19,200,000 shares authorized; 1,725,992 issued

and outstanding.	270,500	17,260
Treasury stock: 677,463 shares held at December 31, 2014	(5,055,906)	—
Additional paid-in capital	309,364,835	809,667
Accumulated deficit	(112,943,613 )	(28,978,824 )
Total stockholders' equity (deficit)	191,635,816	(28,151,897 )
<b>TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY (DEFICIT)</b>	<b>\$ 195,794,240</b>	<b>\$ 14,941,638</b>

The accompanying notes are an integral part of these financial statements.

Bellicum Pharmaceuticals, Inc.

## Statements of Operations

	Year Ended December 31,		
	2014	2013	2012
<b>REVENUES</b>			
Grants	\$ 1,780,523	\$ 1,940,657	\$ 1,470,330
Total revenues	1,780,523	1,940,657	1,470,330
<b>OPERATING EXPENSES</b>			
Research and development	11,008,347	7,049,420	5,796,233
ARIAD license restructuring	43,211,822	—	—
General and administrative	5,398,050	2,813,190	1,943,206
Total operating expenses	59,618,219	9,862,610	7,739,439
<b>LOSS FROM OPERATIONS</b>	<b>(57,837,696 )</b>	<b>(7,921,953 )</b>	<b>(6,269,109)</b>
<b>OTHER INCOME (EXPENSE)</b>			
Interest income	34,975	3,921	7,545
Interest expense	(1,791,173)	(50,719 )	(1,405 )
Change in fair value of warrant liability	(24,370,895)	—	—
Total other income (expense)	(26,127,093)	(46,798 )	6,140
<b>NET LOSS</b>	<b>\$ (83,964,789 )</b>	<b>\$(7,968,751)</b>	<b>\$(6,262,969)</b>
Preferred stock dividends	(1,432,369 )	(1,093,648)	(757,492 )
Net loss attributable to common stockholders	\$ (85,397,158 )	\$(9,062,399)	\$(7,020,461)
Net loss per common share attributable to common shareholders, basic and diluted	\$ (34.04 )	\$(5.25 )	\$(4.26 )
Weighted-average shares outstanding-basic and diluted	2,508,960	1,725,992	1,648,198

The accompanying notes are an integral part of these financial statements.



Bellicum Pharmaceuticals, Inc.

Statements of Redeemable and Convertible Preferred Stock and Stockholders' Equity (Deficit)

Years Ended December 31, 2014, 2013 and 2012

Amount	Series B		Series C		Common Stock		Treasury Stock		Additional Paid-In Capital	A
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount		
3,617	2,174,824	\$10,144,504	—	—	1,357,662	\$13,577	—	—	\$1,984,259	(\$
									91,503	
	675,105	3,122,360							(56,358)	
					368,330	3,683			257,461	
		757,492							(757,492	)
3,617	2,849,929	14,024,356	—	—	1,725,992	17,260	—	—	1,519,373	(\$
									390,595	
	757,497	3,503,426								
	2,955,857	13,670,839							(6,653	)
		1,093,648							(1,093,648)	

33,617	6,563,283	32,292,269	—	—	1,725,992	17,260	—	—	809,667	(
									911,645	
					117,647	1,176			(1,176)	
					12,615	126			11,124	
					8,452,500	84,525			146,217,872	
1,582,706	7,320,015									
		10,091,743	42,074,483							
		6,524,195	72,186,775							
					510,524	5,105			244,596	
	1,432,369								(1,432,369 )	
	(173,744 )									
							(677,463)	(5,055,906)		
33,617	(8,145,989)	(40,870,909)	(16,615,938)	(114,261,258)	16,230,777	162,308			162,603,476	(

\$

\$

— \$— — — 27,050,055 \$270,500 (677,463) (5,055,906) \$309,364,835 \$(

The accompanying notes are an integral part of these financial statements.

Bellicum Pharmaceuticals Inc.

## Statements of Cash Flows

	Year Ended December 31,		
	2014	2013	2012
<b>CASH FLOWS FROM OPERATING ACTIVITIES:</b>			
Net loss	\$(83,964,789 )	\$(7,968,751 )	\$(6,262,969 )
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation expense	666,607	587,248	115,282
Share-based compensation	911,645	390,595	91,503
Stock issued for license agreement	—	—	261,144
Loss on disposal of property and equipment	—	—	3,274
Amortization of lease liability	(89,414 )	(95,500 )	(3,979 )
Interest expense converted into preferred stock	—	3,426	—
Change in fair value of warrant liability	24,370,895	—	—
Changes in operating assets and liabilities:			
Accounts receivable – grants	447,639	(745,541 )	—
Prepaid expenses and other current assets	(1,068,200 )	591,510	(817,597 )
Other assets	339,502	(286,787 )	58,862
Accounts payable	659,412	(1,253 )	59,016
Accrued payroll	259,957	148,999	321,962
Accrued liabilities	(34,600 )	549,483	(215,488 )
Deferred revenue – grants	13,122	(1,038,763 )	(1,470,329 )
Deferred rent	4,627	13,630	63,563
Deferred manufacturing costs	175,819	239,134	52,133
<b>NET CASH USED IN OPERATING ACTIVITIES</b>	<b>(57,307,778 )</b>	<b>(7,612,570 )</b>	<b>(7,743,623 )</b>
<b>CASH FLOWS FROM INVESTING ACTIVITIES:</b>			
Purchases of property and equipment	(804,123 )	(366,338 )	(2,047,483 )
<b>CASH USED IN INVESTING ACTIVITIES</b>	<b>(804,123 )</b>	<b>(366,338 )</b>	<b>(2,047,483 )</b>
<b>CASH FLOWS FROM FINANCING ACTIVITIES:</b>			
Proceeds from issuance of common stock	160,608,750	—	—
Payment of issuance costs on common stock	(14,242,318 )	—	—
Proceeds from issuance of preferred stock	62,320,015	13,670,839	3,122,360
Payment of issuance costs on preferred stock	(3,524,081 )	(6,653 )	(56,358 )
Proceeds from exercise of preferred warrants	39,145,160	—	—
Proceeds from exercise of common warrants	249,701	—	—
Payment for repurchase of common stock	(5,055,906 )	—	—
Payment of preferred dividends	(155,394 )	—	—
Proceeds from notes payable	—	3,500,000	—
Proceeds from line of credit	386,475	550,223	449,777
Payments on line of credit	(1,186,475 )	(200,000 )	—
<b>NET CASH PROVIDED BY FINANCING ACTIVITIES</b>	<b>238,545,927</b>	<b>17,514,409</b>	<b>3,515,779</b>
<b>NET CHANGE IN CASH AND CASH EQUIVALENTS</b>	<b>180,434,026</b>	<b>9,535,501</b>	<b>(6,275,327)</b>
<b>CASH AND CASH EQUIVALENTS AT BEGINNING OF PERIOD</b>	<b>11,167,585</b>	<b>1,632,084</b>	<b>7,907,411</b>
<b>CASH AND CASH EQUIVALENTS AT END OF PERIOD</b>	<b>\$ 191,601,611</b>	<b>\$ 11,167,585</b>	<b>\$ 1,632,084</b>
<b>SUPPLEMENTAL CASH FLOW INFORMATION:</b>			
Cash paid during the period for interest	\$ 1,766,590	\$ 47,296	\$ 788
<b>NON-CASH INVESTING AND FINANCING ACTIVITIES</b>			
Dividends accrued on preferred stock	\$ 1,432,369	\$ 1,093,648	\$ 757,492
Conversion of notes payable into preferred stock	\$ —	\$ 3,500,000	\$ —

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Preferred stock dividends paid in common stock	\$3,195,781	\$—	\$—
Landlord funded leasehold improvements	\$—	\$—	\$413,940

The accompanying notes are an integral part of these financial statements.

Notes to the Financial Statements

NOTE 1 - ORGANIZATION AND BUSINESS DESCRIPTION

Bellicum Pharmaceuticals, Inc. (the Company or Bellicum), was incorporated in Delaware in July 2004 and is based in Houston, Texas. The Company is 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. The Company is devoting substantially all of its present efforts to developing next-generation product candidates in some of the most important areas of cellular immunotherapy, including, hematopoietic stem cell transplantation, CAR T cell therapy and dendritic cell vaccines. The Company has not generated any revenue from product sales to date and does not anticipate generating revenues from product sales in the foreseeable future.

The future success of the Company is dependent on its ability to successfully complete the development of, and obtain regulatory approval for, its product candidates, managing the growth of the organization, obtaining additional financing necessary in order launch and commercialize its products candidates, and competing successfully with other companies in its industry.

NOTE 2 - SIGNIFICANT ACCOUNTING POLICIES

Basis of Presentation

The accompanying financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America. Any reference in these footnotes to applicable guidance is meant to refer to the authoritative U.S. generally accepted accounting principles (GAAP) as found in the Accounting Standards Codification (ASC) and Accounting Standards Update (ASU) of the Financial Accounting Standards Board (FASB).

Revenue Recognition

The Company has not yet generated any revenue from product sales. The Company's sole source of revenue has been grant revenue related to a \$5.7 million research grant received from the Cancer Prevention and Research Institute of Texas (CPRIT), covering a three-year period from July 1, 2011 through June 30, 2014, and a \$0.7 million research grant from the National Institutes of Health (NIH) covering the period from April 2013 to March 2015. Grant payments received prior to the Company's performance of work required by the terms of the research grant are recorded as deferred revenue and recognized as grant revenue once work is performed and qualifying costs are incurred. (See Note 11).

Cash and Cash Equivalents

The Company considers all short-term, highly liquid investments with maturity of three months or less from the date of purchase to be cash equivalents.

Property and Equipment

Leasehold improvements, furniture, equipment and software are recorded at cost and are depreciated using the straight-line method over the estimated useful lives of the related assets, which range from three to five years.

Impairment of Long-Lived Assets

The Company reviews long-lived assets for impairment when events or changes in business conditions indicate that their carrying value may not be recoverable. Recoverability of assets to be held and used is measured by a comparison of the carrying amount of an asset to the estimated undiscounted future cash flows expected to be generated by the asset. If the carrying amount of an asset exceeds its estimated future cash flows, an impairment charge is recognized in the amount by which the carrying amount of the asset exceeds the fair value of the asset. There were no impairment charges related to long-lived assets for the years ended December 31, 2014, 2013 and 2012.

#### Deferred Rent

The Company recognizes rent expense for leases with increasing annual rents on a straight-line basis over the term of the lease. The amount of rent expense in excess of cash payments is classified as deferred rent. Any lease incentives received are deferred and amortized over the term of the lease.

## Notes to the Financial Statements

### Clinical Trials

The Company estimates its clinical trial expense accrual for a given period based on the number of patients enrolled at each site and the length of time each patient has been in the trial, less amounts previously billed.

### Fair Value of Financial Instruments

Accounting standards include disclosure requirements around fair values used for certain financial instruments and establish a fair value hierarchy. The three-tier hierarchy prioritizes valuation inputs into three levels based on the extent to which inputs used in measuring fair value are observable in the market.

Fair value measurements are classified and disclosed in one of the following three categories:

Level 1—Quoted unadjusted prices for identical instruments in active markets.

Level 2—Quoted prices for similar instruments in active markets, quoted prices for identical or similar instruments in markets that are not active and model-derived valuations in which all observable inputs and significant value-drivers are observable in active markets.

Level 3—Model derived valuations in which one or more significant inputs or significant value-drivers are unobservable, including assumptions developed by the Company.

The Company believes the recorded values of their financial instruments, including cash and cash equivalents, accounts receivable, accounts payable, accrued liabilities, and the Series C warrants approximate their fair values due to the short-term nature of these instruments. The carrying amount of the line of credit approximated fair value as it bore interest at variable rates.

### Financial Instruments and Credit Risks

Financial instruments that potentially subject the Company to credit risk include cash and cash equivalents and accounts receivable. Cash is deposited in demand accounts in federally insured domestic institutions to minimize risk. Insurance is provided through the Federal Deposit Insurance Corporation (FDIC) and Security Investor Protection Corporation (SIPC). Although the balances in these accounts exceed the federally insured limit from time to time, the Company has not incurred losses related to these deposits.

### Debt

The Company records proceeds from debt issuances at their face value, less any discounts or the value of any beneficial conversion features or detachable warrants. Interest is accrued over the term of the debt, at the stated interest rate. Discounts are amortized to interest expense through the effective interest method over the term of the debt. Unamortized discounts are immediately recognized as interest expense upon conversion or repayment of the debt.

### Equity Issuance Costs

Equity issuance costs represent costs paid to third parties in order to obtain equity financing. These costs have been netted against the proceeds of the equity issuances.

### Licenses and Patents



Licenses and patent costs for technologies that are utilized in research and development and have no alternative future use are expensed as incurred. Costs related to the license of patents from third parties and internally developed patents are classified as research and development expenses. Legal costs related to patent applications and maintenance are classified as general and administrative expenses.

#### Research and Development

Research and development expenses include salaries for research and development personnel and related payroll expenses, consulting fees, laboratory costs, manufacturing costs for clinical trials, licenses and clinical trial expenses. All costs for research and development are expensed as incurred.

## Notes to the Financial Statements

### Contract Manufacturing Services

Contract manufacturing services are expensed as incurred. Prepaid costs are capitalized and amortized as services are performed.

### Share-Based Compensation

The Company accounts for its share-based compensation in accordance with ASC 718, Compensation — Stock Compensation, which requires the measurement and recognition of compensation expense for all share-based payment awards made to employees and directors to be recognized in the financial statements, based on their fair value. The Company measures share-based compensation to consultants in accordance with ASC 505-50, Equity-Based Payments to Non-Employees, and recognizes the fair value of the award over the period the services are rendered or goods are provided.

The Company uses the Black-Scholes option-pricing model to estimate the fair value of stock option awards. The fair value is recognized as expense, net of estimated forfeitures, over the requisite service period, which is generally the vesting period of the respective award on a straight-line basis. Prior to the Company's initial public offering of its common stock on December 17, 2014, the determination of the grant date fair value of options using the Black-Scholes option-pricing model was affected by the Company's estimated common stock fair value, as well as assumptions regarding a number of other complex and subjective variables.

### Income Taxes

Income taxes are accounted for under the asset and liability method. Deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax bases. This method also requires the recognition of future tax benefits such as net operating loss and tax credit carry forwards, to the extent that realization of such benefits is more likely than not. A valuation allowance is recorded when the realization of future tax benefits is uncertain. The Company records a valuation allowance for the full amount of deferred tax assets, which would otherwise be recorded for tax benefits relating to the operating loss and tax credit carryforwards, as realization of such deferred tax assets cannot be determined to be more likely than not.

Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled. The effect on deferred tax assets and liabilities of a change in tax rates is recognized in the statement of operations in the period that includes the enactment date.

The Company accounts for uncertain tax positions in accordance with the provisions of ASC 740, Income Taxes. When uncertain tax positions exist, the Company recognizes the tax benefit of tax positions to the extent that the benefit will more likely than not be realized. The determination as to whether the tax benefit will more likely than not be realized is based upon the technical merits of the tax position as well as consideration of the available facts and circumstances. As of December 31, 2014 and 2013, the Company had no uncertain tax positions and no interest or penalties have been charged to the Company for the years ended December 31, 2014, 2013 and 2012. If incurred, the Company will classify any interest and penalties as a component of interest expense and operating expense, respectively. The Company is subject to routine audits by taxing jurisdictions; however, there are currently no audits for any tax periods in progress. The tax years 2005 through 2014 remain open to examination by the Internal Revenue Service.

### Comprehensive Income (Loss)

Comprehensive income (loss) is defined as the change in equity of a business enterprise during a period, from transactions, and other events and circumstances from non-owner sources. For the years ended December 31, 2014, 2013 and 2012, net loss equaled comprehensive loss.

### Use of Estimates

The preparation of the financial statements in accordance with GAAP requires management to make certain estimates and judgments that affect the reported amounts of assets, liabilities, and expenses. Actual results could differ from those estimates.

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## Notes to the Financial Statements

Historically, prior to the Company's IPO in December 2014, the fair values of the shares of common stock underlying the Company's share-based awards were estimated on each grant date by its Board of Directors. Given the absence of a public trading market for the Company's common stock, its Board of Directors exercised reasonable judgment and considered a number of objective and subjective factors to determine the best estimate of the fair value of its common stock, including the following:

- its stage of development;
- its operational and financial performance;
- the nature of its services and its competitive position in the marketplace;
- the value of companies that it considers peers based on a number of factors, including similarity to the Company with respect to industry and business model;
- the likelihood of achieving a liquidity event, such as an initial public offering and the nature and history of its business;
- issuances of preferred stock and the rights, preferences, and privileges of its preferred stock relative to those of its common stock;
- business conditions and projections;
- the history of the Company and progress of its research and development efforts and clinical trials; and
- the lack of marketability of its common stock.

## Net Loss and Net Loss per Share of Common Stock Attributable to Common Stockholders

Basic net loss per share attributable to common stockholders is calculated by dividing the net loss attributable to common stockholders by the weighted-average number of share of common stock outstanding during the period without consideration for common stock equivalents. Diluted net loss per share of common stock is the same as basic net loss per share of common stock, since the effects of potentially dilutive securities are antidilutive. The net loss per share of common stock attributable to common stockholders is computed using the two-class method required for participating securities. All series of the Company's convertible preferred stock are considered to be participating securities as they are entitled to participate in undistributed earnings with shares of common stock. Due to the Company's net loss, there is no impact on the earnings per share calculation in applying the two-class method since the participating securities have no legal requirement to share in any losses.

The following outstanding shares of common stock equivalents were excluded from the computations of diluted net loss per shares of common stock attributable to common stockholders for the periods presented as the effect of including such securities would be anti-dilutive.

	December 31, 2014	December 31, 2013	December 31, 2012
Series A Convertible Preferred Stock - as converted to common stock	—	1,496,782	1,496,782
Series B Convertible Preferred Stock - as converted to common stock	—	3,860,754	1,676,428
Warrants to purchase common stock	—	866,570	866,570
Options to purchase common stock	2,733,793	1,574,398	1,067,058
Stock dividends to be issued as payment for Series B dividends	—	101,951	44,393
Total Common Stock Equivalents	2,733,793	7,900,455	5,151,231

## Recently Issued Accounting Pronouncements

In June 2014, the FASB issued Accounting Standards Update No. 2014-12 Compensation – Stock Compensation (ASU No. 2014-12), which clarified the accounting for share-based payments when the terms of an awards provide that a performance target could be achieved after the requisite service period. ASU No. 2014-12 is effective for annual reporting purposes beginning after December 15, 2015, with early adoption allowed. The Company does not believe ASU No. 2014-12 will have a material effect on its financial statements.

## Notes to the Financial Statements

In August 2014, the FASB issued Accounting Standard Update No. 2014-15, Presentation of Financial Statements—Going Concern (Subtopic 205-40), (ASU No. 2014-15), which requires management to assess an entity's ability to continue as a going concern by incorporating and expanding upon certain principles that are currently in U.S. auditing standards. Specifically, ASU 2014-15 provides a definition of the term substantial doubt and requires an assessment for a period of one year after the date that the financial statements are issued (or available to be issued). It also requires certain disclosures when substantial doubt is alleviated as a result of consideration of management's plans and requires an express statement and other disclosures when substantial doubt is not alleviated. ASU No. 2014-15 is effective for fiscal years, and interim periods within those years, beginning after December 15, 2016, and early application is permitted.

The Company has evaluated other recent accounting pronouncements and believes that none of them will have a material effect on the Company's financial statements.

**NOTE 3 - CASH AND CASH EQUIVALENTS**

As of December 31, 2014 and 2013, the Company invested approximately \$43.6 million and \$10.9 million, respectively, in cash equivalent instruments.

**NOTE 4 - FAIR VALUE OF FINANCIAL INSTRUMENTS**

ASC 820, Fair Value Measurement, provides a comprehensive framework for measuring the fair value of assets and liabilities, which provides for consistency in how fair value determinations are made under various existing accounting standards that permit, or in some cases require, estimates of fair market value.

Financial assets and liabilities that have recurring fair value measurements are shown below:

	Balance at	Quoted prices in active markets for identical assets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
	December 31, 2014			
<b>Assets:</b>				
Money market funds	\$ 43,586,791	\$ 43,586,791	\$ —	\$ —
<b>Total</b>	<b>\$ 43,586,791</b>	<b>\$ 43,586,791</b>	<b>\$ —</b>	<b>\$ —</b>

	Balance at	Quoted prices in active markets for identical assets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
	December 31, 2013			
<b>Assets:</b>				
Money market funds	\$ 10,879,656	\$ 10,879,656	\$ —	\$ —
<b>Total</b>	<b>\$ 10,879,656</b>	<b>\$ 10,879,656</b>	<b>\$ —</b>	<b>\$ —</b>

## NOTE 5 - PROPERTY AND EQUIPMENT

Property and equipment consists of the following:

Description	Estimated Useful Lives	December 31, 2014	December 31, 2013
Lab equipment	5 years	\$ 1,716,741	\$ 1,133,305
Office furniture	5 years	334,607	326,595
Software	3 years	75,235	47,890
Computer equipment	3 to 5 years	205,191	151,182
Leasehold improvements	5 years	1,506,322	1,375,001
Total		3,838,096	3,033,973
Less: accumulated depreciation		(1,410,661 )	(744,054 )
		\$ 2,427,435	\$ 2,289,919

During the years ended December 31, 2014, 2013 and 2012, the Company recorded \$0.7 million, \$0.6 million and \$0.1 million of depreciation expense, respectively.

## Notes to the Financial Statements

## NOTE 6 - ACCRUED LIABILITIES

Accrued liabilities consist of the following:

	December 31, 2014	December 31, 2013
Commission on exercise of warrants	\$730,719	\$—
Medical facility fees	200,594	224,166
Patient treatment costs	128,139	197,713
License costs	50,000	175,000
Other	321,973	67,294
Total accrued liabilities	\$1,431,425	\$664,173

## NOTE 7 - DEBT

## Promissory Notes – 2013

On February 12, 2013, the Company received \$3.5 million of cash proceeds through the issuance of promissory notes, bearing interest at 0.21% per annum from February 12, 2013 through July 31, 2013. On July 31, 2013, in connection with the issuance of Series B Preferred Stock, the Company repaid the notes with 757,497 shares of Series B preferred convertible redeemable stock at a conversion price of \$4.625 per share. The repaid balance consisted of \$3.5 million of principal and \$3,426 of outstanding interest payable.

## Line of Credit

The Company was party to a line of credit which was executed in December 2012 for \$1.0 million. The annual interest rate was equal to the prime rate plus 2.75%. The Company took advances under this line of credit to fund equipment purchases and other capital expenditures. During 2012, the Company was advanced \$0.5 million under the line of credit. During 2013, the Company was advanced \$0.6 million and repaid \$0.2 million in principal under the line of credit. Principal payments on the \$1.0 million line of credit began in July 2013 and were to be paid over 30 months. During 2014, the line of credit was amended to include a credit extension up to \$0.5 million. Interest accrued at a rate of prime plus 2.75% from the date of each advance. Any advances that were outstanding on the credit extension were payable in 24 equal monthly installments of principal, plus all accrued interest, beginning on April 1, 2015. During the year ended December 31, 2014, \$0.4 million was advanced under the credit extension. During 2014, the Company repaid the \$1.2 million in principal outstanding and interest of \$34,318. The line of credit was paid off as of December 31, 2014.

## NOTE 8 - DEFERRED MANUFACTURING COSTS

On December 5, 2011, the Company entered into a service agreement with a third party to perform manufacturing processes for the Company's products. The agreement contained the following terms: (i) an initial non-refundable commitment fee of \$1.0 million; (ii) a monthly fee of \$91,200 which will be deducted from the initial commitment fee until it is fully depleted; (iii) a minimum of 110 batches at a cost of \$3,400 per batch plus the cost of materials to be manufactured over a minimum of 18 months, commencing October 2012.



The Company recorded the commitment fee as prepaid manufacturing costs and expensed the monthly fee as research and development costs to offset against the prepaid manufacturing costs. As of December 31, 2014 and 2013, all of the prepaid manufacturing costs had been recognized.

The minimum 110 batches represent the minimum quantity that the Company has to procure. If the Company elects to terminate the agreement before 110 batches are produced, the Company must pay \$3,400 per batch for the production shortfall. The Company initially expected to fulfill this requirement in 18 months, commencing October 2012. Accordingly, the Company accrued the value of the 110 batches ratably over the 18 months as deferred manufacturing costs. Subsequently in 2014, the Company revised its estimate of the expected term of the agreement to extend an additional 21 months. As of December 31, 2014 and 2013, the deferred manufacturing costs were \$0.3 million in each year.

Additionally, the third party manufacturer agreed to credit Bellicum \$0.3 million against its monthly fees beginning April 1, 2014. This credit would be forfeited if the agreement were terminated prior to April 1, 2014. This credit was applied to the monthly fee beginning April 1, 2014 at a rate of \$3,000 per day. The Company is recognizing the credit ratably over the remaining expected term of the agreement as a reduction of research and development expenses commencing April 2014. As of December 31, 2014 the balance of the credit was \$0.2 million.

Notes to the Financial Statements

NOTE 9 - Common Stock, Preferred Stock and Warrants

Common Stock

As of December 31, 2014 and 2013, the Company had 200,000,000 and 19,200,000, respectively, authorized shares of common stock with a par value of \$0.01 per share.

Exercise of Common Warrants

In March 2014, the Company issued 393,523 shares of common stock for \$200,700, or \$0.51 per share in conjunction with the exercise of warrants expiring in March of 2014.

In December 2014, the Company issued 117,001 shares of common stock for \$49,001, or \$0.42 per share in conjunction with the exercise of warrants expiring in 2015.

Reverse Stock Split

On December 4, 2014, the Company's board of directors and stockholders approved an amendment to the Company's amended and restated certificate of incorporation to effect a reverse split of shares of the Company's common stock on a 1-for-1.7 basis (the "Reverse Stock Split"). The par value and the authorized shares of the common stock were not adjusted as a result of the Reverse Stock Split. All issued and outstanding common stock, options for common stock, warrants for common stock, and per share amounts contained in the financial statements have been retroactively adjusted to reflect this Reverse Stock Split for all periods presented.

Initial Public Offering

On December 17, 2014, the Company commenced its initial public offering (IPO) pursuant to a registration statement on Form S-1 (File No. 333- 200328) that was declared effective by the SEC on December 17, 2014 and that registered an aggregate of 7,350,000 shares of the Company's common stock for sale to the public at a price of \$19.00 per share. In addition, at the closing of the initial public offering on December 23, 2014, the underwriters exercised their over-allotment option to purchase 1,102,500 additional shares of the Company's common stock in the initial public offering at the public offering price of \$19.00 per share, for an aggregate offering price of \$160.6 million. The net offering proceeds to the Company, after deducting underwriting discounts, commissions and offering costs, were approximately \$146.3 million.

Treasury Stock

In December 2014, in conjunction with the restructuring of the license agreement with ARIAD, the Company acquired 677,463 shares of its Common Stock valued at approximately \$5.1 million. See Note 12.

Preferred Stock

As of December 2014, the Company had 10,000,000 authorized shares of preferred stock. There were no preferred shares issued or outstanding at December 31, 2014. As of December 2013 the Company had 11,700,000 authorized shares of convertible redeemable preferred stock with a par value of \$0.01 per share.

The Company had shares of two classes of convertible redeemable preferred stock issued and outstanding as of December 31, 2013: Series A convertible redeemable preferred stock (Series A) and Series B convertible redeemable preferred stock (Series B), each with a par value of \$0.01. The shares of Series A were issued between March 2009 and November 2011 at a price of \$3.00 per share. The shares of Series B were issued between November 2011 and January 2014 at a price of \$4.625 per share. On August 22, 2014, the Company filed Amended and Restated Articles of Incorporation with which the Company was authorized to issue Series C convertible preferred stock, and certain changes were made to the rights, preferences and privileges of Series A and Series B.

On August 22, 2014, the Company issued 10,091,743 shares of Series C convertible preferred stock (Series C) at a purchase price of \$5.45 per share and warrants to purchase up to 6,559,598 shares of Series C with an exercise price of \$6.00 per share. The warrants had a five year term, but were subject to earlier termination in the event of a Qualified IPO (defined in the warrant agreement), on or prior to March 31, 2015, or upon a merger or sale of the Company. The Company received net proceeds from the issuance of Series C preferred stock of \$51.5 million, net of offering costs of \$3.5 million. Series A, Series B and Series C are collectively referred to herein as the Preferred Stock. All of the Preferred Stock was converted to common stock at the time of the IPO, and no shares of Preferred Stock remained outstanding at December 31, 2014.

Notes to the Financial Statements

The following table summarizes the Company's Preferred Shares for the three years ended December 31, 2014:

	Series A			Series B			Series C		
	Initial Shares	Initial Value	Redemption Value	Initial Shares	Initial Value	Redemption Value	Initial Shares	Initial Value	Redempt Value
ending y 1,	2,544,539	\$7,633,617	\$7,633,617	2,174,824	\$10,058,559	\$10,144,504	—	\$—	\$—
ce of B ed or et	—	—	—	675,105	3,122,360	3,122,360	—	—	—
ion es B nds					-	757,492			
ending ber 2	2,544,539	\$7,633,617	\$7,633,617	2,849,929	\$13,180,919	\$14,024,356	—	\$—	\$—
ce of B ed or et	—	—	—	2,955,857	13,670,839	13,670,839	—	—	—
ersion and t into B ed				757,497	3,503,426	3,503,426			
ion es B nds					-	1,093,648			
ending ber 3	2,544,539	\$7,633,617	\$7,633,617	6,563,283	\$30,355,184	\$32,292,269	—	\$—	\$—
ce of B ed or et	—	—	—	1,582,706	7,320,015	7,320,015			
ce of C ed							10,091,743	42,074,483	42,074,

or									
et									
ce of									
C									
ed									
n									
ce of									
ts							6,524,195	72,186,775	72,186,
ion									
es B									
nds							1,432,369		
nt of									
B									
nds							(173,744	)	
rsion									
mon									
	(2,544,539)	(7,633,617)	(7,633,617)	(8,145,989)	(37,675,199)	(40,870,909)	(16,615,938)	(114,261,258)	(114,26
nding									
ber									
4	—	\$—	\$—	—	\$—	\$—	—	\$—	\$—

The rights, preferences and privileges of the Preferred Stock, as of December 31, 2014 and 2013, are as follows:

#### Optional Conversion

Each share of Series A, Series B and Series C was convertible, at the option of the holder at any time and without additional consideration, into one share of common stock. The Series A, Series B and Series C conversion price was \$3.00 per share, \$4.625 per share and \$5.45 per share, respectively. The rate at which shares of Preferred Stock may be converted into shares of common stock, was subject to anti-dilution protection in the event of certain dilutive issuances of capital stock.

#### Mandatory Conversion

Upon the closing of the sale of the Company's common stock in an IPO at a price per share of at least \$6.50 (as adjusted for splits, dividends and the like) and resulting in at least \$50.0 million of gross proceeds to the Company, all of the outstanding shares of Preferred Stock will automatically convert into shares of the Company's common stock, at the then-applicable conversion rate, and such shares may not be reissued by the Company. As of December 31, 2014 and 2013, cumulative dividends were \$0 and \$1.9 million, respectively.

Upon the closing of the IPO, all of the outstanding shares of Preferred Stock automatically converted into shares of the Company's common stock, which were simultaneously reverse split on a 1:1.17 ratio. There were no shares of Preferred Stock outstanding at December 31, 2014.

#### Dividends

Through August 22, 2014, the holders of Series B were entitled to receive an annual dividend, payable quarterly, if, as and when declared, and if not paid, accrued, equal to 6% of the Series B original issue price, subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to Series B. This dividend was cumulative, but not compounded. No dividends or other distributions could be declared or paid with respect to the Series A or common stock, other than dividends payable solely in common stock, unless and

until all dividends due on Series B have been paid or declared and set apart for payment. On August 22, 2014, the Series B dividend was made non-cumulative and cumulative Series B dividends of \$3.4 million were declared and paid in December 2014 in conjunction with the IPO. Subsequent to August 22, 2014, the holders of Preferred Stock were entitled to 8% non-cumulative dividends per annum payable only when, as and if declared by the Board of Directors of the Company.

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Notes to the Financial Statements

Liquidation

At December 31, 2013, in the event of any deemed liquidation event or any voluntary or involuntary liquidation, dissolution, or winding up of the Company, the holders of Series B then outstanding were entitled to be paid out of the assets of the Company available for distribution to its stockholders, before any payment would be made to the holders of Series A or common stock, an amount per share equal to the original issue price of the Series B, plus any dividends accrued or declared but unpaid thereon (Liquidation Amount). On August 22, 2014, the liquidation rights of the holders of Series B and Series A were made subordinate to the holders of Series C.

In the event of any deemed liquidation event, which is defined in the Company's certificate of incorporation to include (1) a merger or consolidation of the Company in which the Company or a subsidiary is a constituent party (subject to certain exceptions), (2) the sale, lease, transfer, exclusive license or other disposition of all or substantially all of the Company's assets, or (3) the sale of outstanding shares of the capital stock of the Company representing at least 50% of the outstanding capital stock or at least 50% of the voting power of the outstanding capital stock (subject to certain exceptions), or any voluntary or involuntary liquidation, dissolution, or winding up of the Company, the holders of Series C then outstanding were entitled to be paid out of the assets of the Company available for distribution to its stockholders, before any payment would be made to the holders of Series A, B or common stock, an amount per share equal to the original issue price of the Series C, plus any dividends declared but unpaid thereon (Liquidation Amount).

After payment to the holders of Series C of the full amounts due them, the holders of Series B and then the holders of Series A were entitled to be paid out of the remaining assets of the Company available for distribution to its stockholders, before any payment would be made to the holders of common stock, in an amount per share equal to the original issue price of the Series A, plus any dividends declared but unpaid thereon.

After payment of the preferential amounts discussed above, the holders of shares of Series A, Series B and Series C, the remaining assets of the Company available for distribution to its stockholders shall be distributed among the holders of the shares of common stock and Preferred Stock, pro rata on an as-converted basis based on the number of shares held by each holder.

Redemption

At December 31, 2013, at any time following the seventh anniversary of the original issue date of the Series B, the Company was obligated to redeem all of the outstanding shares of Series B, if requested in writing to do by the holders of not less than 51% of the outstanding shares of Series B. At any time following the seventh anniversary of the original issue date of the Series A, the Company was obligated to redeem all of the outstanding shares of Series A, if requested in writing to do so by the holders of not less than 51% of the outstanding shares of Series A. No redemption of any of the shares of Series A could occur until such a time as no shares of Series B were outstanding. Effective August 22, 2014, Series A and B became redeemable only upon a deemed liquidation event, which is defined in the Company's certificate of incorporation to include (1) a merger or consolidation of the Company in which the Company or a subsidiary is a constituent party (subject to certain exceptions), (2) the sale, lease, transfer, exclusive license or other disposition of all or substantially all of the Company's assets, or (3) the sale of outstanding shares of the capital stock of the Company representing at least 50% of the outstanding capital stock or at least 50% of the voting power of the outstanding capital stock (subject to certain exceptions).

The effect of the modification of Series A and B features resulted in the stock no longer being probable of becoming redeemable. As such, beginning on August 22, 2014, Series A and B will not be accreted to redemption value until such time as the shares are probable of being redeemable.

The Series C is not redeemable except in a deemed liquidation event. The Company has determined Series C is not probable of becoming redeemable, as such Series C will not be accreted to redemption value until such time as the shares are probable of being redeemable.

#### Voting

Each holder of outstanding shares of Preferred Stock was entitled to cast the number of votes equal to the number of whole shares of common stock into which the shares of Preferred Stock held by the holder were convertible. Except as provided by law or by the other provisions of the Company's Certificate of Incorporation, holders of Preferred Stock vote together with the holders of common stock as a single class.



## Notes to the Financial Statements

## Warrants

## Series C Warrants

In connection with the August 2014 issuance of Series C convertible preferred stock, Bellicum issued warrants to purchase 6,559,598 shares of Series C convertible preferred stock with an exercise price of \$6.00 per share which were convertible into 3,858,549 common shares. The fair value of the warrants on the date of issuance of \$9.4 million, as determined using the Black-Scholes option-pricing model, was recorded as a warrant liability. The Series C warrants were revalued both at September 30, 2014 to \$10.6 million, and again at the time of exercise in December 2014 to \$33.8 million. The increase in the calculated fair value from the issuance date to the remeasurement dates required the recording of a non-cash charge of \$24.4 million in 2014, of which \$23.2 million was a fourth quarter expense. Subsequent to the IPO in December 2014, 6,524,195 of the Series C warrants were exercised and converted into 3,837,727 shares of common stock. The Company received cash proceeds of approximately \$39.2 million as a result of the Series C warrant exercise. Holders of 35,403 Series C warrants chose not to exercise their warrants, and those warrants have expired.

Inputs used to determine estimated fair value (Level 3) include the estimated fair value of the warrant, as determined by the fair value of the underlying stock relative to the warrant exercise price at the valuation measurement date, volatility of the price of the underlying stock, the expected term of the warrants, risk-free interest rates and expected dividends.

The fair value of the warrants has been estimated, with the following assumptions at each measurement date:

	August 22, 2014		September 30, 2014		December 17, 2014	
Volatility	88.6	%	88.6	%	0	%
Risk-free interest rate	0.53	%	0.58	%	0.46	%
Expected dividend yield	0	%	0	%	0	%
Expected life (Years)	1.93		1.82		—	

## Warrants to Purchase Common Stock – 2009 and 2010 Notes Payable

The following table outlines the warrants outstanding and exercisable as of December 31, 2013 and 2012:

	Exercise		Expiration	
Issuance Date	Price	Outstanding	Exercisable	Date
March 2009	\$ 0.51	393,523	393,523	March 15, 2014
September 2010	\$ 0.51	46,072	46,072	September 30, 2015
December 2010	\$ 0.51	71,570	71,570	December 16, 2015

All of the 2009 and 2010 Notes Payable Warrants were exercised or forfeited during 2014 and none remain outstanding at December 31, 2014.

Warrant to Purchase Common Stock – Texas Emerging Technology Fund (TETF)

The Texas Emerging Technology Fund, or TETF, holds a warrant to purchase up to 355,392 shares of the Company's common stock at an exercise price of \$0.0017 per share. These were issued in conjunction with a grant in 2007. This warrant remains outstanding and exercisable indefinitely.

NOTE 10 - Share Based Compensation Plans

The Company has four share-based compensation plans, which authorize the granting of shares of common stock and options to purchase common stock to employees and directors of the Company, as well as non-employee consultants, and allows the holder of the option to purchase common stock at a stated exercise price. Options vest according to the terms of the grant, which may be immediately or based on the passage of time, generally over four years, and have a term of up to 10 years. Unexercised stock options terminate on the expiration date of the grant. The Company recognizes the share-based compensation expense over the requisite service period of the individual grantees, which generally equals the vesting period.

Notes to the Financial Statements

2014 Employee Stock Purchase Plan

The Company adopted the 2014 Employee Stock Purchase Plan (the ESPP) in December 2014, pursuant to which Eligible Employees of the Company, as defined by the ESPP may be given an opportunity to purchase up to 550,000 shares of Common Stock, reserved for issuance under the ESPP, through payroll deductions. The Plan permits the Company to grant a series of Purchase Rights to Eligible Employees under the ESPP.

The Company's Board of Directors may from time to time grant or provide for the grant of Purchase Rights to Eligible Employees under an Offering (consisting of one or more Purchase Periods) on an Offering Date or Offering Dates selected by the Board. Each Offering will be in such form and will contain such terms and conditions as the Board will deem appropriate, and will comply with the requirement of Section 423(b)(5) of the Internal Revenue Code of 1986, as amended, including any applicable regulations and guidance thereunder (the Code) that all Employees granted Purchase Rights will have the same rights and privileges. The terms and conditions of an Offering shall be incorporated by reference into the Plan and treated as part of the Plan. The provisions of separate Offerings need not be identical, but each Offering will include (through incorporation of the provisions of this Plan by reference in the document comprising the Offering or otherwise) the period during which the Offering will be effective, which period will not exceed 27 months beginning with the Offering Date.

The purchase price of shares of Common Stock acquired pursuant to Purchase Rights will be not less than the lesser of:

- i. an amount equal to 85% of the Fair Market Value of the shares of Common Stock on the Offering Date; or
  - ii. an amount equal to 85% of the Fair Market Value of the shares of Common Stock on the applicable Purchase Date.
- The Company will expect to record share-based compensation expense to the extent that shares are purchased for less than their Fair Market Value under the ESPP. No funds were withheld from employees during 2014, and the Company did not incur any share-based compensation cost under the ESPP.

2014 Equity Incentive Plan

Under the 2014 Equity Incentive Plan, 5,582,970 shares of the Company's authorized but unissued common stock were reserved for issuance to employees, consultants, or to non-employee members of the Board. The 2014 Equity Incentive Plan replaced the Company's previous stock option plan (the 2011 Stock Option Plan). There were 141,176 options, and 117,647 shares of restricted common stock outstanding at December 31, 2014 under this plan.

2011 Stock Option Plan

Under the 2011 Stock Option Plan, 2,798,500 shares of the Company's authorized but unissued common stock were reserved for issuance to employees, consultants, or to non-employee members of the Board. The 2011 Stock Option Plan replaced the 2006 Stock Option Plan. There were 2,425,561 and 1,407,343 outstanding options under this plan at December 31, 2014 and 2013, respectively. As of December 31, 2014 there were no additional shares available for grant under this plan. During 2014, a total of 12,615 options were exercised for cash of \$11,250. The Company did not realize tax benefits from the exercise.

2006 Stock Option Plan

Under the 2006 Stock Option Plan, as amended, 177,352 shares of the Company's authorized but unissued common stock were reserved for issuance to optionees, including officers, employees, and other individuals performing

services for the Company. A total of 167,056 options were outstanding under this plan as of December 31, 2014 and 2013. As of December 31, 2014, there were no additional shares available for grant under the 2006 Stock Option Plan.

The valuation of the share-based compensation awards is a significant accounting estimate that requires the use of judgment and assumptions that are likely to have a material impact on the financial statements. The fair value of option grants is determined using the Black-Scholes option-pricing model. Expected volatilities utilized in the model are based on implied volatilities from traded stocks of peer companies. Similarly, the dividend yield is based on historical experience and the estimate of future dividend yields. The risk-free interest rate is derived from the U.S. Treasury yield curve in effect at the time of grant. The expected term of the options is based on the average period the stock options are expected to remain outstanding. As the Company does not have sufficient historical information to develop reasonable expectations about future exercise patterns and post-vesting employment termination behavior, the expected term is calculated as the midpoint between the weighted-average vesting term and the contractual expiration period also known as the simplified method. The Company assumed no awards would be forfeited during the vesting period, as actual forfeitures

## Notes to the Financial Statements

have been minimal through December 31, 2014. The fair value of the option grants have been estimated, with the following weighted-average assumptions:

	December 31, 2014	December 31, 2013	December 31, 2012
Volatility	95 %	90 %	75 %
Risk-free interest rate	1.86 %	1.58 %	1.97 %
Expected dividend yield	0 %	0 %	0 %
Expected life (Years)	6.09	6.25	6.25

A summary of the status of the Company's stock option plans as of December 31, 2014 and changes from December 31, 2013 through December 31, 2014 is as follows:

	Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term (Years)
Options outstanding, December 31, 2013	1,574,398	\$ 2.33	8.26
Options granted	1,188,806	8.67	—
Options exercised	(12,615 )	2.55	—
Options forfeited	(16,796 )	2.55	—
Options outstanding, December 31, 2014	2,733,793	\$ 5.09	8.39
Options Exercisable, December 31, 2014	1,265,326	\$ 2.28	7.11

A summary of the Company's restricted share activity for the year ended December 31, 2014 is as follows:

	Restricted Shares	Weighted-Average Grant Date Fair Value
Restricted shares outstanding at December 31, 2013	—	—
Shares granted	117,647	\$ 19.00
Shares vested	—	—
Shares forfeited	—	—
Restricted shares outstanding at December 31, 2014	117,647	\$ 19.00

The following table includes share based payment activity for the years ended December 31, 2014, 2013 and 2012:

	Year Ended December		
	31, 2014	2013	2012
Weighted-average grant date fair value of options granted	\$13.30	\$1.38	\$0.31
Weighted-average grant date fair value of restricted shares granted	\$19.00	\$—	\$—
Aggregate intrinsic value of options exercised	\$58,900	\$—	\$—
Cash received by Company upon option exercises	\$11,250	\$—	\$—

The Company calculates the intrinsic value of its options by multiplying the number of options by the difference between the estimated fair value per share for its common stock and the options' exercise price. The aggregate intrinsic value of options exercisable and options outstanding at December 31, 2014 was \$26.3 million and \$49.1 million, respectively. The Company will issue new shares of common stock upon the exercise of vested options.

## Notes to the Financial Statements

The following table outlines the options outstanding and exercisable as of December 31, 2014, 2013 and 2012:

## Options Outstanding at December 31, 2014

Exercise Price	Options	Aggregate Intrinsic Value	Weighted-Average
			Remaining Contractual Term (Years)
\$0.34	5,882	\$133,521	1.76
0.51	161,174	3,631,250	5.89
2.55	1,417,636	29,047,362	7.50
7.47	1,007,925	15,693,392	9.86
19.00	141,176	570,351	9.97
Total			
Outstanding		2,733,793	\$49,075,877 8.39

## Options Exercisable at December 31, 2014

Exercise Price	Options	Aggregate Intrinsic Value	Weighted-Average
			Remaining Contractual Term (Years)
\$0.34	5,882	\$133,521	1.76
0.51	161,174	3,631,250	5.89
2.55	1,098,270	22,503,552	7.32
Total			
Exercisable		1,265,326	\$26,268,324 7.11

## Options Outstanding at December 31, 2013

Exercise Price	Options	Aggregate Intrinsic Value	Weighted-Average
			Remaining Contractual Term (Years)
\$0.34	5,882	\$10,646	2.76
0.51	161,176	264,325	6.89
2.55	1,407,340	—	8.44
Total			
Outstanding		1,574,398	\$274,972 8.26

## Options Exercisable at December 31, 2013

Exercise Price	Options	Intrinsic Value	Aggregate
			Weighted-Average Remaining Contractual Term (Years)
\$0.34	5,882	\$ 10,646	2.76
0.51	137,212	222,058	6.89
2.55	675,586	—	8.25
<b>Total</b>			
Exercisable		818,680	\$ 232,704 7.98

## Options Outstanding at December 31, 2012

Exercise Price	Options	Intrinsic Value	Aggregate
			Weighted-Average Remaining Contractual Term (Years)
\$0.34	5,882	\$ 2,118	3.76
0.51	161,176	30,623	7.89
2.55	900,000	—	8.89
<b>Total</b>			
Outstanding		1,067,058	\$ 32,741 8.71

## Options Exercisable at December 31, 2012

Exercise Price	Options	Intrinsic Value	Aggregate
			Weighted-Average Remaining Contractual Term (Years)
\$0.34	5,882	\$ 2,118	3.76
0.51	109,565	\$ 20,480	7.90
2.55	381,023	—	8.88
<b>Total</b>			
Exercisable		496,470	\$ 22,598 8.60



## Notes to the Financial Statements

Share-based compensation expense for the year ended December 31, 2014, 2013 and 2012 was \$0.9 million, \$0.4 million, and \$0.1 million, respectively. At December 31, 2014, total compensation cost not yet recognized was \$17.6 million and the weighted average period over which this amount is expected to be recognized is 3.64 years. The aggregate fair value of options vesting in the years ended December 31, 2014, 2013 and 2012 was \$0.3 million, \$0.3 million and \$0.1 million, respectively.

## NOTE 11 - GRANT REVENUE

## CPRIT Grant

On July 27, 2011, the Company entered into a Cancer Research Grant Contract (Grant Contract) with the Cancer Prevention and Research Institute of Texas (CPRIT) under which CPRIT awarded a grant not to exceed approximately \$5.7 million to be used by the Company for the execution of defined clinical development of BPX-501. In addition, CPRIT could award supplemental funding not to exceed ten percent of the total grant amount based upon the Company's progress. The Grant Contract terminated on June 30, 2014. The terms of the Grant Contract require the Company to pay tiered royalties on revenues from sales and licenses of intellectual property facilitated by the Grant Contract.

During 2014, 2013, and 2012, the Company incurred \$1.4 million, \$1.8 million and \$1.5 million of expenses under the Grant Contract, respectively. As of December 31, 2014 and 2013, the Company had an outstanding grant receivable of \$0.3 million and \$0.7 million, respectively, for grant expenditures that were paid but had not yet been reimbursed.

## NIH Grant

During 2014 and 2013, the Company was awarded \$0.3 million and \$0.4 million, respectively, under a grant from the National Institutes of Health (NIH). The awards cover the period from April 2013 through March 2015. The awards were made pursuant to the authority of 42 USC 241 42 CFR 52, and is subject to the requirements of the statute. Funds spent on the grant are reimbursed through monthly reimbursement requests.

As of December 31, 2014 and 2013 funds spent under the grant were \$0.3 million and \$0.2 million, respectively. As of December 31, 2014, the Company had deferred revenue of \$13,000 related to the grant. As of December 31, 2013, the Company had outstanding grant receivables of \$29,000.

## NOTE 12 - ARIAD LICENSE RESTRUCTURING

On March 7, 2011, the Company entered into an amended and restated exclusive license agreement with ARIAD (Amended ARIAD License) which amended a license agreement entered into by the parties in 2006. Under the Amended ARIAD License, ARIAD granted to the Company an exclusive (even as to the ARIAD) license, with the right to grant sublicenses, under ARIAD's patent rights relating to dimerizers, genetic constructs coding for dimerizer binding domains, vectors containing said constructs, cells containing said constructs and methods of inducing biological processes in cells containing said constructs. These licensed patent rights were initially limited to the fields of cell transplantation and certain types of cancer.

In connection with the initial license, in 2006, the Company issued 121,241 shares of its common stock to ARIAD which were subject to antidilution protection that ultimately resulted in additional issuances to ARIAD by the Company of 556,222 shares of the Company's common stock, such that ARIAD received a total of 677,463 shares of common stock under the license agreement. In addition, the Company paid ARIAD a license fee of \$250,000 in

connection with the amendment in 2011. The Amended ARIAD license also provided for certain royalty and milestone payments, which were subsequently terminated pursuant to an omnibus amendment agreement with ARIAD.

Under the Amended ARIAD License, the Company is required to diligently proceed with the development, manufacture and sale of licensed products. The Amended ARIAD License is subject at all times to restrictions and obligations under a license agreement by and between ARIAD Gene Therapeutics, Inc. (one of ARIAD's affiliates which merged into ARIAD) and the academic institution from which ARIAD obtained its license to the underlying technology. While the Company is not required to pay royalties or fees to such academic institution, no sublicensee of the Company's may enter into a sublicense with respect to any intellectual property owned by the academic institution without its consent, which terms must be consistent with those included in the agreement between ARIAD and such academic institution.

The Amended ARIAD License will expire upon expiration of the last license term of a licensed product covered by the agreement, which is either the later of (i) 12 years from the date of the first commercial sale of the licensed product, or (ii) expiration of a valid claim on the licensed product. Either party to the license may terminate or modify the Amended ARIAD License upon a material

## Notes to the Financial Statements

breach by the other party 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 bankruptcy of the other party. The Company may terminate the amended ARIAD license in its sole discretion at any time if the Company determines not to develop or commercialize any licensed product. In addition, upon termination of the amended ARIAD license prior to expiration, the Company must transfer any ownership and any beneficial ownership in any orphan drug designation or any similar designation in any jurisdiction of orphan drug status of the ARIAD dimerizer to ARIAD.

On October 3, 2014, the Company entered into an omnibus amendment agreement with ARIAD, under which the Company agreed to make payments of \$50.0 million in exchange for an expansion of the license field, the termination of all obligations to make milestone and royalty payments to ARIAD in the future and the return of 677,463 shares of common stock held by ARIAD.

In connection with the amendment, the Company made an initial payment of \$15.0 million and issued a promissory note to ARIAD for a principal amount of \$35.0 million. Per the promissory note terms, the principal would not accrue interest unless the Company was in default, in which case it would accrue at a rate of 10% per annum. In December, 2014, following the Company's IPO, the Company paid the remaining \$35.0 million and ARIAD returned all 677,463 shares of common stock of the Company that ARIAD held. The license transaction was valued on the date of the transaction and the note was discounted to fair market value at a 10% rate. This resulted in the ARIAD license expense of \$43.2 million, repurchase of common stock for \$5.1 million and interest expense of \$1.7 million. The Company has recorded the returned shares of common stock as treasury stock.

## NOTE 13 - COMMITMENTS AND CONTINGENCIES

## Leases

Prior to December 2012, the Company had entered into various short-term leases for office space. The Company incurred rent expense during the years ended December 31, 2014, 2013 and 2012 of \$0.3 million, \$0.2 million and \$0.2 million, respectively.

In December 2012, the Company entered into a five-year office lease agreement. During 2013, the lease was amended to include additional space. During 2014, the lease was amended again to include additional space and extend the term of the lease. After the 2013 and 2014 amendments, the leased premises totals 35,250 square feet. The lease includes escalating base rent payments, which initially increased on November 1, 2013, and then increased again on December 1, 2013. An additional base rent increase will become effective February 1, 2015 as a result of the 2014 lease modification. Subsequently, an increase in the base rent payment will occur during the first month of each year, and remain constant for the next 11 months. During the last month of each year, the monthly base rent payment will increase yet again. This escalating base rent payment structure will continue through the expiration of the lease on January 31, 2020.

The future minimum payments under the lease are as follows:

Year	Minimum Lease Obligation
2015	\$ 980,625
2016	1,049,428
2017	1,067,053

2018	1,084,679
2019	1,104,531
Thereafter	93,268
Total Minimum lease payments	\$ 5,379,584

## Litigation

The Company, from time to time, may be involved in litigation relating to claims arising out of its ordinary course of business. Management believes that there are no material claims or actions pending or threatened against the Company.

## License Agreements with Baylor College of Medicine

### 2008 Baylor License Agreement

Pursuant to an Exclusive License Agreement with Baylor College of Medicine (Baylor), dated March 20, 2008 (2008 Baylor License), the Company obtained an exclusive, worldwide and fully paid up license to certain intellectual property, including intellectual

## Notes to the Financial Statements

property related to methods for activating antigen presenting cells and to genetic constructs coding for membrane bound inducible cytoplasmic CD40.

As consideration for the 2008 Baylor License, the Company issued to Baylor 23,529 shares of its common stock and assumed responsibility for all legal fees and expenses, filing or maintenance fees, assessments and all other costs and expenses related to prosecuting, obtaining and maintaining patent protection on the patents subject to the 2008 Baylor License.

The 2008 Baylor License is subject to certain restrictions and is nonexclusive with respect to (i) the making or use of the licensed intellectual property for use in non-commercial research, patient care, teaching, and other educational purposes; (ii) any non-exclusive license covering the licensed intellectual property that Baylor grants to other academic or research institutions for noncommercial research purposes; and (iii) any non-exclusive licenses that Baylor is required to grant to the U.S. or foreign state pursuant to an existing or future treaty with the U.S., and (iv) a non-exclusive license granted to ARIAD under the terms of a materials transfer agreement between Baylor and ARIAD.

Baylor may terminate or modify the 2008 Baylor License in the event of a material breach 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. The Company may terminate the 2008 Baylor License, or any portion thereof, at its sole discretion at any time upon 30 days' written notice to Baylor. Upon termination of the 2008 Baylor License, all rights to the intellectual property immediately revert to Baylor.

### 2010 Baylor License Agreement

Pursuant to an Exclusive License Agreement with Baylor, dated June 27, 2010 (2010 Baylor License), the Company obtained an exclusive, worldwide license to certain intellectual property, including intellectual property related to methods for treating prostate cancer, methods of administering T cells to a patient, and methods of activating antigen presenting cells with constructs comprising MyD88 and CD40.

Pursuant to the terms of the 2010 Baylor License, the Company paid Baylor a license execution fee of \$30,000. In addition, the Company is required to pay a low annual maintenance fee on each anniversary of the agreement date.

The terms of the 2010 Baylor License also require the Company to make royalty payments of less than one percent, subject to certain annual minimums, on net sales of products covered by the license. In addition, to the extent the Company enters into a sublicensing agreement relating to a licensed product, the Company is required to pay Baylor a percentage in the mid-single digits on all non-royalty income received from sublicensing revenue. The Company is required to make milestone payments, of up to \$0.7 million in aggregate, upon successful completion of clinical and regulatory milestones regarding the first two product covered by this license.

The 2010 Baylor License will expire upon expiration of the last patent contained in the licensed patent rights, on a country-by-country basis, upon which the Company will have a perpetual, paid-in-full license in such country. Baylor may terminate or modify the 2010 Baylor License in the event of a material breach by the Company 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. The Company may terminate the 2010 Baylor License, or any portion thereof, at the Company's sole discretion at any time upon 60 days' written notice to Baylor. Upon termination of the 2010 Baylor License for any reason prior to expiration, the

Company must assign to Baylor each authorized sublicense agreement that is currently in effect on the date of termination.

#### 2014 Baylor License Agreement

Pursuant to an Exclusive License Agreement with Baylor, effective November 1, 2014, or the 2014 Baylor license agreement, we obtained an exclusive, worldwide license to certain intellectual property, including intellectual property related to methods for inducing selective apoptosis.

Pursuant to the terms of the 2014 Baylor license agreement, we paid Baylor a license execution fee of \$25,000. In addition, we are required to pay Baylor a low annual maintenance fee on each anniversary of the agreement date. The terms of the 2014 Baylor license agreement also require us to make royalty payments in the low single digits, subject to certain annual minimums, on net sales of products covered by the license. To the extent we enter into a sublicensing agreement relating to a licensed product, we are also required to pay Baylor a percentage in the low double-digits on all non-royalty income received from sublicensing revenue. We are required to make milestone payments, of up to \$0.3 million in aggregate, upon successful completion of clinical and regulatory

## Notes to the Financial Statements

milestones regarding the first product covered by this license. The 2014 Baylor license agreement will expire upon expiration of the last patent contained in the licensed patent rights, on a country-by-country basis, upon which we will have a perpetual, paid-in-full license in each such country.

Baylor may terminate or modify the 2014 Baylor license agreement 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. We may terminate the 2014 Baylor license agreement, or any portion thereof, at our sole discretion at any time upon 60 days' written notice to Baylor.

## Employment agreements

The Company has signed agreements with seven of its officers and key employees to provide certain benefits in the event of a "change of control" as defined in these agreements and the occurrence of certain other events. The agreements provide for a lump-sum payment in cash equal to 12 to 18 months of annual base salary and annual cash bonus, if any. The annual base salary and annual cash bonus portion of the agreements would aggregate approximately \$3.3 million at the rate of compensation in effect at December 31, 2014. In addition, the agreements provide for continuation of certain insurance and other benefits for periods of 12 to 18 months.

## NOTE 14 - RELATED PARTY TRANSACTIONS

## Related Party Licensing Arrangements

During 2010, the Company entered into a license agreement with Baylor College of Medicine, granting the Company the rights to patents and technology derived from invention disclosure BLG 06-028. The license included \$30,000 of upfront payments during 2010 and 2011, and additional annual fees of \$7,500 beginning on the second anniversary of the license agreement.

During 2008, Bellicum entered into a license agreement with Baylor College of Medicine, granting the Company the rights to patents and technology derived from invention disclosures OTA 01-085 and BLG 08-024. Consideration for the license included issuance in 2009 of 13,529 common shares to Baylor College of Medicine and 5,882 common shares to the inventors (including board members Dr. David Spencer and Dr. Kevin Slawin).

On March 1, 2006, the Company issued 61,764 common shares to Dr. David Spencer, Dr. Brent Hanks and Dr. Kevin Slawin, valued at \$0.34 per share, as consideration for the use of certain patents. During 2006, the Company issued 121,241 common shares to ARIAD as consideration for the licensed rights to use certain patents held by ARIAD. The stock purchase agreement included certain anti-dilutive features which resulted in the issuance of the following additional shares:

Year:	Anti-Dilutive Shares Issued
2009	187,891
2012	368,330

## NOTE 15 - INCOME TAXES

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The Company did not recognize tax expense during 2014, 2013 or 2012. The reconciliation between federal income taxes at the statutory rate and the Company's income tax expense for the year is as follows:

	December 31, 2014	December 31, 2013	December 31, 2012
U.S. tax benefit at statutory rate	\$(28,548,028)	\$(2,709,375)	\$(2,129,409)
Meals and entertainment	9,534	3,721	3,711
Incentive stock options	97,764	115,020	13,329
Warrant expense	8,286,104	—	—
Deferred tax valuation allowances	20,586,161	3,027,513	2,112,369
Research and development credit	(431,535 )	(436,879 )	—
Income tax expense	\$—	\$—	\$—

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## Notes to the Financial Statements

Deferred income taxes reflect the net tax effect of temporary differences between the carrying amounts of assets and liabilities for financial reporting purposes, and the amounts used for income tax purposes. Significant components of the Company's deferred taxes as of December 31, 2014 and 2013 are as follows:

	2014	2013
Deferred tax liabilities:		
Depreciation	\$(127,238 )	\$(194,887 )
Prepaid costs	(188,850 )	(123,486 )
Tenant improvement allowance	(83,271 )	(111,419 )
Total deferred tax liabilities	(399,359 )	(429,792 )
Deferred tax assets:		
Net operating loss carry-forward	14,044,251	9,141,814
Nonqualified stock options	268,123	71,761
Restricted stock expense	15,833	—
Tenant improvement liability	96,059	128,528
Deferred contract manufacturing costs	161,121	99,030
Research and development credit	1,257,696	826,161
ARIAD license restructuring	14,977,254	
Other	7,336	4,652
Total deferred tax assets:	30,827,673	10,271,946
Valuation allowance	(30,428,314)	(9,842,154)
Total deferred tax	\$—	\$—
Net current deferred tax liability	\$(174,703 )	\$(64,000 )
Net non-current deferred tax asset	174,703	64,000
Total deferred tax	\$—	\$—

As of December 31, 2014 and 2013, the Company had gross federal income tax net operating loss (NOL) carry forwards of \$41.3 million and \$26.9 million, respectively, and federal research tax credits of \$1.3 million and \$0.8 million, respectively. The net operating loss carry-forwards will expire beginning in 2024, if not utilized. In assessing the realization of deferred tax assets, management considers whether it is more likely than not that some portion or all of the deferred tax asset will be realized. The ultimate realization of deferred tax assets is dependent upon the Company attaining future taxable income during periods in which those temporary differences become deductible. Due to the uncertainty surrounding the realization of the benefits of its deferred assets, including net operating loss carry forwards, the Company has provided a 100% valuation allowance on its deferred tax assets at December 31, 2014 and 2013. The increases in the valuation allowance were \$20.6 million and \$3 million for the years ended December 31, 2014 and 2013, respectively.

The Internal Revenue Code Section 382 limits net operating loss and tax credit carry forwards when an ownership change of more than 50% of the value of the stock in a loss corporation occurs. Accordingly, the ability to utilize remaining net operating loss and tax credit carry forwards may be significantly restricted.



## Notes to the Financial Statements

## NOTE 16 - QUARTERLY FINANCIAL DATA (UNAUDITED)

The following is a summary of the unaudited quarterly results of operations (in thousands, except per-share data).(1)

	Unaudited Year Ended December 31, 2014			
	Quarter	Quarter	Quarter	Quarter
	Ended	Ended	Ended	Ended
	March 31	June 30	September 30	December 31 (2)
Total revenues	\$552	\$553	\$ 660	\$ 15
Loss from operations	(2,277)	(3,273)	(2,898 )	(49,390 )
Net loss	\$(2,290)	\$(3,281)	\$(4,097 )	\$(74,298 )
Preferred dividends	\$(540 )	\$(564 )	\$(328 )	\$—
Net loss attributable to common shareholders	\$(2,830)	\$(3,845)	\$(4,425 )	\$(74,298 )
Net loss per share attributable to common shareholders - basic and diluted	\$(1.52 )	\$(1.81 )	\$(2.08 )	\$(18.99 )

	Unaudited Year Ended December 31, 2013			
	Quarter	Quarter	Quarter	Quarter
	Ended	Ended	Ended	Ended
	March 31	June 30	September 30	December 31
Total revenues	\$183	\$218	\$ 721	\$ 819
Loss from operations	(1,772)	(1,773)	(1,895 )	(2,482 )
Net loss	\$(1,782)	\$(1,786)	\$(1,908 )	\$(2,493 )
Preferred dividends	\$(195 )	\$(197 )	\$(303 )	\$(399 )
Net loss attributable to common shareholders	\$(1,977)	\$(1,983)	\$(2,211 )	\$(2,892 )
Net loss per share attributable to common shareholders - basic and diluted	\$(1.15 )	\$(1.15 )	\$(1.28 )	\$(1.68 )

(1)The amounts were computed independently for each quarter, and the sum of the quarters may not total the annual amounts.

(2)The quarter results include ARIAD license restructuring of \$43.2 million and the change in fair value of the warrant liability of \$23.2 million.

ITEM 9. Changes in and Disagreements with Accountants on Accounting and Financial Disclosure

None.

ITEM 9A. Controls and Procedures

Evaluation of Disclosure Controls and Procedures.

We are responsible for maintaining disclosure controls and procedures, as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act. Disclosure controls and procedures are controls and other procedures designed to ensure that the information required to be disclosed by us in the reports that we file or submit under the Exchange Act is recorded, processed, summarized, and reported within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by us in the reports that we file or submit under the Exchange Act is accumulated and communicated to our management, including our principal executive officer and our principal financial officer, as appropriate to allow timely decisions regarding required disclosure.

As of December 31, 2014, we carried out an evaluation, under the supervision and with the participation of our management, including our chief executive officer and principal financial officer, of the effectiveness of the design and operation of our disclosure controls and procedures, as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act. Based on this evaluation, our chief executive officer and principal financial officer concluded that our disclosure controls and procedures were not effective at the reasonable assurance level as of December 31, 2014, as the additional controls and processes that we implemented between June 30, 2014 and December 31, 2014 were not complete and were not in place for a sufficient period of time for us to assess their effectiveness in remediating the material weakness as of December 31, 2014. The material weaknesses in our internal controls over financial reporting that we identified mid-year and for which we have initiated remediation measures relate to (1) a lack of internal controls over accounting and financial reporting, particularly surrounding nonroutine transactional and financial reporting, (2) a lack of sufficient staff, including, at the time, the lack of a chief financing officer or other senior finance executive and (3) a lack of formalized accounting policy and procedure documentation that is followed by accounting personnel.

Management's Report on Internal Control over Financial Reporting.

This Annual Report does not include a report of management's assessment regarding internal control over financial reporting or an attestation report of the Company's registered public accounting firm due to a transition period established by rules of the SEC for newly public companies.

Changes in Internal Control over Financial Reporting.

There were no changes in our internal control over financial reporting during the quarter ended December 31, 2014 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

ITEM 9B. Other Information

None.



### PART III

#### ITEM 10. Directors, Executive Officers and Corporate Governance

The information required by this item and not set forth below will be set forth in the sections entitled “Election of Directors,” “Executive Officers” and “Section 16(a) Beneficial Ownership Reporting Compliance” in our definitive proxy statement for our 2015 Annual Meeting of Stockholders, or our Proxy Statement, to be filed with the SEC within 120 days after the end of the fiscal year ended December 31, 2014, and is incorporated herein by reference.

We have adopted a written code of business conduct and ethics that applies to our directors, officers and employees, including our principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions. A current copy of the code is posted on the Corporate Governance section of our website, which is located at [www.bellicum.com](http://www.bellicum.com). If we make any substantive amendments to, or grant any waivers from, the code of business conduct and ethics for any officer or director, we will disclose the nature of such amendment or waiver on our website or in a current report on Form 8-K.

#### ITEM 11. Executive Compensation

The information required by this item will be set forth in the section entitled “Executive Compensation” in our Proxy Statement and is incorporated herein by reference.

#### ITEM 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters

The information required by this item will be set forth in the section headed “Security Ownership of Certain Beneficial Owners and Management” in our Proxy Statement and is incorporated herein by reference.

The information required by Item 201(d) of Regulation S-K will be set forth in the section headed “Executive Compensation” in our Proxy Statement and is incorporated herein by reference.

#### ITEM 13. Certain Relationships and Related Party Transactions, and Director Independence

The information required by this item will be set forth in the sections entitled “Certain Relationships and Related Party Transactions” and “Election of Directors” in our Proxy Statement and is incorporated herein by reference.

#### ITEM 14. Principal Accounting Fees and Services

The information required by this item will be set forth in the section entitled “Principal Accounting Fees and Services” in our Proxy Statement and is incorporated herein by reference.

(a) Exhibits

Exhibit Number	Description
3.1	Amended and Restated Certificate of Incorporation of the Registrant (incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K, filed with the SEC on December 23, 2014).
3.2	Amended and Restated Bylaws of the Registrant (incorporated by reference to Exhibit 3.2 to the Registrant's Current Report on Form 8-K, filed with the SEC on December 23, 2014).
4.1	Reference is made to Exhibits 3.1 and 3.2.
4.2	Form of Common Stock Certificate of the Registrant (incorporated by reference to Exhibit 4.1 to the Registrant's Registration

- Statement on Form S-1, as amended (File No. 333-200328), originally filed with the SEC on November 18, 2014).
- 4.3 Second Amended and Restated Investor Rights Agreement by and among the Registrant and certain of its stockholders, dated August 22, 2014 (incorporated by reference to Exhibit 4.2 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-200328), originally filed with the SEC on November 18, 2014).
- 4.4 Warrant to Purchase Common Stock issued to the State of Texas, dated September 27, 2007 (incorporated by reference to Exhibit 4.5 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-200328), originally filed with the SEC on



November 18,  
2014).

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and between the  
Registrant and its  
directors and  
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10.2+ Bellicum  
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originally filed  
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2014).

10.3+ Bellicum  
Pharmaceuticals,  
Inc. 2011 Stock  
Option Plan and  
Forms of

	<p>Incentive Stock Option Grant Agreement and Nonqualified Stock Option Agreement (incorporated by reference to Exhibit 10.3 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-200328), originally filed with the SEC on November 18, 2014).</p>
10.4+	<p>Bellicum Pharmaceuticals, Inc. 2014 Equity Incentive Plan and Form of Stock Option Grant Notice, Stock Option Agreement and Notice of Exercise thereunder (incorporated by reference to Exhibit 10.4 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-200328), originally filed with the SEC on November 18, 2014).</p>
10.5+	<p>Bellicum Pharmaceuticals, Inc. Non-Employee Director</p>

	<p>Compensation Policy (incorporated by reference to Exhibit 10.5 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-200328), originally filed with the SEC on November 18, 2014).</p>
10.6+	<p>Third Amended and Restated Employment Agreement by and between the Registrant and Thomas J. Farrell, dated November 17, 2014 (incorporated by reference to Exhibit 10.6 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-200328), originally filed with the SEC on November 18, 2014).</p>
10.7+	<p>Amended and Restated Employment Agreement by and between the Registrant and David M. Spencer, Ph.D., dated November 17, 2014</p>

(incorporated by reference to Exhibit 10.7 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-200328), originally filed with the SEC on November 18, 2014).

10.8+

Employment Agreement by and between the Registrant and Annemarie Moseley Ph.D., M.D., dated October 17, 2011 (incorporated by reference to Exhibit 10.8 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-200328), originally filed with the SEC on November 18, 2014).

10.9+

Amendment 1 to Employment Agreement by and between the Registrant and Annemarie Moseley Ph.D., M.D., dated October 17, 2011 (incorporated by reference to Exhibit 10.9 to the Registrant's Registration Statement on

Form S-1, as amended (File No. 333-200328), originally filed with the SEC on November 18, 2014).

10.10+

Third Amended and Restated Consulting Agreement by and between the Registrant and Kevin M. Slawin, M.D., dated November 9, 2011 (incorporated by reference to Exhibit 10.10 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-200328), originally filed with the SEC on November 18, 2014).

Exhibit Number	Description
10.11+	Employment Offer Letter by and between the Registrant and Ken Moseley, J.D., dated December 6, 2011 (incorporated by reference to Exhibit 10.11 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-200328), originally filed with the SEC on November 18, 2014).
10.12+	Employment Agreement by and between the Registrant and Joseph H. Senesac, dated November 16, 2014 (incorporated by reference to Exhibit 10.12 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-200328), originally filed with the SEC on November 18, 2014).
10.13+	Employment Agreement by and between the Registrant and Peter L. Hoang, dated November 17, 2014 (incorporated by reference to Exhibit 10.13 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-200328), originally filed with the SEC on November 18, 2014).

- 10.14 Notice of Expansion of Licensed Field to Obtain Additional Exclusive Rights (incorporated by reference to Exhibit 10.14 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-200328), originally filed with the SEC on November 18, 2014).
- 10.15\* Amended and Restated License Agreement by and between the Registrant and ARIAD Pharmaceuticals, Inc., dated March 7, 2011 (incorporated by reference to Exhibit 10.15 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-200328), originally filed with the SEC on November 18, 2014).
- 10.16\* Omnibus Amendment Agreement by and between Registrant and ARIAD Pharmaceuticals, Inc., dated October 3, 2014 (incorporated by reference to Exhibit 10.16 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-200328), originally filed with the SEC on November 18, 2014).

- 10.17\* Exclusive License Agreement by and between the Registrant and Baylor College of Medicine, dated March 20, 2008 (incorporated by reference to Exhibit 10.17 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-200328), originally filed with the SEC on November 18, 2014).
- 10.18\* Exclusive License Agreement by and between the Registrant and Baylor College of Medicine, dated June 27, 2010 (incorporated by reference to Exhibit 10.18 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-200328), originally filed with the SEC on November 18, 2014).
- 10.19\* Cancer Research Grant Contract by and between the Registrant and the Cancer Prevention and Research Institute of Texas, dated July 27, 2011 (incorporated by reference to Exhibit 10.19 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-200328), originally filed with the SEC on November 18, 2014).



- 10.20\* Exclusive License Agreement by and between the Registrant and Baylor College of Medicine, effective November 1, 2014 (incorporated by reference to Exhibit 10.20 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-200328), originally filed with the SEC on November 18, 2014).
- 10.21 Lease Agreement by and between Registrant and Sheridan Hills Developments L.P., dated June 1, 2012 (incorporated by reference to Exhibit 10.21 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-200328), originally filed with the SEC on November 18, 2014).
- 10.22 First Amendment to Lease Agreement by and between Registrant and Sheridan Hills Developments L.P., dated September 13, 2013 (incorporated by reference to Exhibit 10.22 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-200328), originally filed with the SEC on November 18, 2014).

- 10.23                      Second Amendment to Lease Agreement by and between Registrant and Sheridan Hills Developments L.P., dated June 20, 2014 (incorporated by reference to Exhibit 10.23 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-200328), originally filed with the SEC on November 18, 2014).
- 10.24                      Third Amendment to Lease Agreement by and between Registrant and Sheridan Hills Developments L.P., dated July 21, 2014 (incorporated by reference to Exhibit 10.24 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-200328), originally filed with the SEC on November 18, 2014).
- 10.25                      Fourth Amendment to Lease Agreement by and between Registrant and Sheridan Hills Developments L.P., dated November 12, 2014 (incorporated by reference to Exhibit 10.25 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-200328), originally filed with the SEC on November 18, 2014).

10.26

Loan and Security Agreement by and between the Registrant and Comerica Bank, dated December 13, 2012 (incorporated by reference to Exhibit 10.27 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-200328), originally filed with the SEC on November 18, 2014).

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Exhibit Number	Description
10.28	First Amendment to Loan and Security Agreement by and between the Registrant and Comerica Bank, dated March 1, 2014 (incorporated by reference to Exhibit 10.28 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-200328), originally filed with the SEC on November 18, 2014).
10.29	Second Amendment to Loan and Security Agreement by and between the Registrant and Comerica Bank, dated July 3, 2014 (incorporated by reference to Exhibit 10.29 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-200328), originally filed with the SEC on November 18, 2014).
10.30	Employment Agreement by and between the Registrant and Alan A. Musso, dated December 4, 2014 (incorporated by reference to Exhibit 10.30 to the Registrant's Registration Statement

on Form S-1, as amended (File No. 333-200328), originally filed with the SEC on November 18, 2014).

- 23.1 Consent of Ernst & Young LLP, an Independent Registered Public Accounting Firm.
- 24.1 Power of Attorney. Reference is made to the signature page hereto.
- 31.1 Certification of the Principal Executive Officer pursuant to Rule 13a-14(a) or 15d-14(a) of the Securities Exchange Act of 1934.
- 31.2 Certification of the Principal Financial Officer pursuant to Rule 13a-14(a) or 15d-14(a) of the Securities Exchange Act of 1934.
- 32.1 Certification of the Principal Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
- 32.2 Certification of the Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.

- + Indicates management contract or compensatory plan.
- \* Confidential treatment has been granted with respect to certain portions of this exhibit. Omitted portions have been filed separately with the SEC.

## SIGNATURES

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

Bellicum Pharmaceuticals, Inc.

Date: March 20, 2015 By: /s/ Thomas J. Farrell  
Thomas J. Farrell

President and Chief Executive Officer

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints Thomas J. Farrell as his true and lawful attorney-in-fact, with the power of substitution, for him in any and all capacities, to sign any amendments to this report, and to file the same, with exhibits thereto and other documents in connection therewith with the Securities and Exchange Commission, hereby ratifying and confirming all that each of said attorney-in-fact, or his substitute or substitutes may do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the Registrant in the capacities and on the dates indicated:

Signature	Title	Date
/s/ Thomas J. Farrell Thomas J. Farrell	President, Chief Executive Officer and Member of the Board of Directors (Principal Executive Officer)	March 20, 2015
/s/ Alan A. Musso Alan A. Musso	Chief Financial Officer and Treasurer (Principal Financial and Accounting Officer)	March 20, 2015
/s/ Kevin M. Slawin, M.D. Kevin M. Slawin, M.D.	Chief Technology Officer and Member of the Board of Directors	March 20, 2015
/s/ James Brown James Brown	Chairman of the Board of Directors	March 20, 2015
/s/ Reid M. Huber, Ph.D Reid M. Huber, Ph.D	Member of the Board of Directors	March 20, 2015
/s/ Frank B. McGuyer Frank B. McGuyer	Member of the Board of Directors	March 20, 2015

Frank B. McGuyer

/s/ Jon P. Stonehouse

Member of the Board of Directors

March 20,  
2015

Jon P. Stonehouse



INDEX TO EXHIBITS

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10.2+ Bellicum  
Pharmaceuticals,  
Inc. 2006 Stock  
Option Plan and  
Form of  
Nonqualified  
Stock Option  
Agreement  
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Exhibit 10.2 to  
the Registrant's  
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10.3+ Bellicum  
Pharmaceuticals,  
Inc. 2011 Stock  
Option Plan and

Forms of  
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10.4+ Bellicum  
Pharmaceuticals,  
Inc. 2014 Equity  
Incentive Plan  
and Form of  
Stock Option  
Grant Notice,  
Stock Option  
Agreement and  
Notice of  
Exercise  
thereunder  
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Exhibit 10.4 to  
the Registrant's  
Registration  
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10.5+ Bellicum  
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Inc.  
Non-Employee

Director  
Compensation  
Policy  
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Exhibit 10.5 to  
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and Restated  
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and between the  
Registrant and  
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Farrell, dated  
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and between the  
Registrant and  
David M.  
Spencer, Ph.D.,  
dated November

17, 2014  
(incorporated by  
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the Registrant's  
Registration  
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10.8+ Employment  
Agreement by  
and between the  
Registrant and  
Annemarie  
Moseley Ph.D.,  
M.D., dated  
October 17, 2011  
(incorporated by  
reference to  
Exhibit 10.8 to  
the Registrant's  
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10.9+ Amendment 1 to  
Employment  
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and between the  
Registrant and  
Annemarie  
Moseley Ph.D.,  
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and Restated  
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and between the  
Registrant and  
Kevin M. Slawin,  
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10.28	First Amendment to Loan and Security Agreement by and between the Registrant and Comerica Bank, dated March 1, 2014 (incorporated by reference to Exhibit 10.28 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-200328), originally filed with the SEC on November 18, 2014).
10.29	Second Amendment to Loan and Security Agreement by and between the Registrant and Comerica Bank, dated July 3, 2014 (incorporated by reference to Exhibit 10.29 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-200328), originally filed with the SEC on November 18, 2014).
10.30	Employment Agreement by and between the Registrant and Alan A. Musso, dated December 4, 2014 (incorporated by reference to Exhibit 10.30 to the Registrant's Registration Statement on Form S-1, as

amended (File No. 333-200328), originally filed with the SEC on November 18, 2014).

- 23.1 Consent of Ernst & Young LLP, an Independent Registered Public Accounting Firm.
- 24.1 Power of Attorney. Reference is made to the signature page hereto.
- 31.1 Certification of the Principal Executive Officer pursuant to Rule 13a-14(a) or 15d-14(a) of the Securities Exchange Act of 1934.
- 31.2 Certification of the Principal Financial Officer pursuant to Rule 13a-14(a) or 15d-14(a) of the Securities Exchange Act of 1934.
- 32.1 Certification of the Principal Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
- 32.2 Certification of the Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.

- + Indicates management contract or compensatory plan.
- \* Confidential treatment has been granted with respect to certain portions of this exhibit. Omitted portions have been filed separately with the SEC.