

BELLICUM PHARMACEUTICALS, INC  
Form 10-K  
March 13, 2017

---

UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
Washington, D.C. 20549  
Form 10-K

(Mark One)

☒ ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934  
For the fiscal year ended December 31, 2016

OR

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

For the transition period from \_\_\_\_\_ to \_\_\_\_\_.  
Commission file number 001-36783

Bellicum Pharmaceuticals, Inc.  
(Exact name of registrant as specified in its charter)

Delaware 20-1450200  
(State or other jurisdiction of incorporation or organization) (I.R.S. Employer Identification No.)

2130 W. Holcombe Blvd., Ste. 800, Houston, TX 77030  
(Address of principal executive offices) (Zip Code)  
(832) 384-1100  
(Registrant's telephone number, including area code)

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

Edgar Filing: BELLICUM PHARMACEUTICALS, INC - Form 10-K

incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K. x

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

☒ x

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 ☒ x

The approximate aggregate market value of the voting and non-voting common equity held by non-affiliates of the Registrant, based upon the last sale price of the common stock reported on The NASDAQ Global Market as of June 30, 2016 was \$251,404,832. \*

As of February 28, 2017, there were 27,157,680 shares of the Registrant's common stock, par value \$0.01 per share, outstanding.

**DOCUMENTS INCORPORATED BY REFERENCE**

Portions of the Registrant's definitive Proxy Statement relating to its 2017 Annual Meeting of Stockholders are incorporated by reference into Part III of this Annual Report on Form 10-K. Such Proxy Statement will be filed with the U.S. Securities and Exchange Commission within 120 days following the Registrant's fiscal year ended December 31, 2016.

---

\*Excludes 7,644,2070 shares of common stock held by directors and officers and by stockholders that the registrant concluded were affiliates of the Registrant as of June 30, 2016. Exclusion of such shares should not be construed to indicate that any such holder possesses the power, direct or indirect, to direct or cause the direction of the management or policies of the Registrant, or that such person is controlled by or under common control with the Registrant.

---

---

Table of Contents

BELLICUM PHARMACEUTICALS, INC.

Form 10-K

For the Fiscal Year Ended December 31, 2016

TABLE OF CONTENTS

PART I

Item 1.	<u>Business</u>	<u>3</u>
Item 1A.	<u>Risk Factors</u>	<u>27</u>
Item 1B.	<u>Unresolved Staff Comments</u>	<u>56</u>
Item 2.	<u>Properties</u>	<u>56</u>
Item 3.	<u>Legal Proceedings</u>	<u>56</u>
Item 4.	<u>Mine Safety Disclosures</u>	<u>56</u>

PART II

Item 5.	<u>Market for Registrant's Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities</u>	<u>57</u>
Item 6.	<u>Selected Financial Data</u>	<u>59</u>
Item 7.	<u>Management's Discussion and Analysis of Financial Condition and Results of Operations</u>	<u>60</u>
Item 7A.	<u>Quantitative and Qualitative Disclosures About Market Risk</u>	<u>71</u>
Item 8.	<u>Financial Statements and Supplementary Data</u>	<u>71</u>
Item 9.	<u>Changes in and Disagreements With Accountants on Accounting and Financial Disclosure</u>	<u>96</u>
Item 9A.	<u>Controls and Procedures</u>	<u>96</u>
Item 9B.	<u>Other Information</u>	<u>97</u>

PART III

Item 10.	<u>Directors, Executive Officers and Corporate Governance</u>	<u>98</u>
Item 11.	<u>Executive Compensation</u>	<u>98</u>
Item 12.	<u>Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters</u>	<u>98</u>
Item 13.	<u>Certain Relationships and Related Transactions, and Director Independence</u>	<u>98</u>
Item 14.	<u>Principal Accounting Fees and Services</u>	<u>98</u>

PART IV

Item 15.	<u>Exhibits, Financial Statement Schedules</u>	<u>99</u>
----------	--	-----------

Signatures

Table of Contents

SPECIAL NOTE REGARDING 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 Chemical Induction of Dimerization, or CID, CID-based technologies, including CaspaCIDE and GoCAR-T;
- 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 and the success of any such collaborations;
- 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, or U.S., 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 cash and other resources; and
- our expectations regarding our ability to obtain and maintain intellectual property protection for our product candidates.

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.

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.

## Table of Contents

### 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. We are using our proprietary CID technology platform to engineer our product candidates with switch technologies that are designed to 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, chimeric antigen receptor T cell therapy, or CAR T, and T cell receptors, or TCRs. HSCT, also known as bone marrow transplantation, has for decades been curative for many patients with hematological cancers or orphan inherited blood disorders. However, adoption of HSCT to date has been limited by the risks of transplant-related morbidity and mortality from graft-versus-host-disease, or GvHD, and the potential for serious infections due to the lack of an effective immune system following a transplant. CAR T and TCR cell therapies are an innovative approach in which a patient's T cells are genetically modified to carry chimeric antigen receptors, or CARs, or TCRs 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," or CRS, neurologic toxicities and cases in which they have attacked healthy organs. In each case, these toxicities have sometimes resulted in 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 that raise even greater safety concerns.

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, 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 and/or persistence of the immunotherapy cells. Each of our product candidates incorporates one of these switches, for enhanced, real time control of safety and efficacy:

CaspaCIDE is our safety switch, incorporated into our HSCT and TCR product candidate, where it is inactive unless the patient experiences a serious side effect. In that event, rimiducid is administered to induce Caspase-9, or iCaspase, switch activation to fully or partially eliminate the cells, with the goal of terminating or attenuating the therapy and resolving the serious side effect.

Our "Go" switch incorporated into our GoCAR-T product candidates, is an activation switch designed to allow control of the activation and proliferation of the 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, reducing the dosage per infusion, or suspending further rimiducid administration.

In addition, we have an active research effort to develop other advanced molecular switch approaches, including a "dual-switch" that is designed to provide a user-controlled system for managing persistence and safety of tumor antigen-specific CAR T cells.

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 lead clinical product candidate is described below.

BPX-501 is a CaspaCIDE product candidate designed as an adjunct T cell therapy administered after allogeneic HSCT. BPX-501 is designed to improve transplant outcomes by enhancing the recovery of the immune system following an HSCT procedure. BPX-501 addresses the risk of infusing donor T cells by enabling the elimination of donor T cells through the activation of the CaspaCIDE safety switch if there is an emergence of uncontrolled GvHD.

The European Commission has granted orphan drug designations to BPX-501 for treatment in HSCT, and for activator agent rimiducid for the treatment of GvHD. Additionally, BPX-501 and rimiducid have received orphan drug status from the U.S. Food and

## Table of Contents

Drug Administration, or the FDA, as a combination replacement T-cell therapy for the treatment of immunodeficiency and GvHD after allogeneic HSCT.

During 2016, we discussed with the European Medicines Agency, or the EMA, clinical and regulatory plans to support the filing of Marketing Authorization Applications, or MAAs, for BPX-501 and rimiducid in Europe, initially for pediatric patients with certain orphan inherited blood disorders or treatment-refractory hematological cancers. Based on the regulatory discussions, we believe that data from the European arm of our BP-004 trial, expanded to enroll additional patients, with a primary endpoint of event-free survival (death, severe GvHD and severe infection) at six months, could form the basis of MAAs for BPX-501 and rimiducid. In addition, the EMA's Committee for Medicinal Products for Human Use, or the CHMP, has agreed that review and approval under "exceptional circumstances" may be suitable, recognizing that a randomized trial may not be feasible in the pediatric haploidentical hematopoietic stem cell transplant setting. Exceptional circumstances may be granted for medicines that treat very rare diseases, or where controlled studies are impractical or not consistent with accepted principles of medical ethics. In place of a randomized trial, we intend to collect data from a concurrent observational study in the pediatric matched unrelated donor hematopoietic stem cell transplant setting, which will include both retrospective patients and prospective patients.

We have discussions ongoing with the FDA regarding the regulatory path to approval in the U.S. and we expect to provide updates in the first half of 2017.

In addition to BPX-501, our clinical stage product candidates which are designed to overcome limitations of CAR T and TCR therapies, include the following:

BPX-701 is a CaspaCIDE-enabled natural high affinity TCR product candidate designed to target malignant cells expressing the preferentially-expressed antigen in melanoma, or PRAME. Initial planned indications for BPX-701 development are refractory or relapsed acute myeloid leukemia, or AML, and myelodysplastic syndromes, or MDS, with an additional study planned for metastatic uveal melanoma. Each of these is an orphan indication where PRAME is highly expressed and for which current treatment options are limited. A Phase 1 dose finding clinical trial in patients with relapsed or refractory myeloid neoplasms is being conducted at the Oregon Health & Science University Hospital in Portland, Oregon.

BPX-601 is a GoCAR-T product candidate containing our proprietary inducible MyD88/CD40, or iMC, activation switch, designed to treat solid tumors expressing prostate stem cell antigen, or PSCA. Preclinical data shows enhanced T cell proliferation, persistence and in vivo anti-tumor activity compared to traditional CAR T therapies. A Phase 1 clinical trial in patients with non-resectable pancreatic cancer is being conducted at the Baylor Sammons Cancer Center in Dallas, Texas.

We have developed an efficient and scalable process to manufacture genetically modified T cells of high quality, which is currently being used by our third-party contract manufacturers to produce BPX-501 for our clinical trials. We are leveraging this process, as well as our resources, capabilities and expertise for the manufacture of our CAR T and TCR product candidates.

## Pipeline

The following table summarizes our product candidate pipeline:



Table of Contents

## 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.

The following therapeutic applications of cellular immunotherapy have been 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 chronic myeloid leukemia. 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.

**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:

## Cellular

Immunotherapy Approach	Safety Challenges	Efficacy Challenges
Allogeneic HSCT	GvHD and viral infections are frequent and potentially fatal side effects	Attempts to control GvHD (steroids, T Cell depletion, etc.) increase likelihood of non-engraftment, relapse of underlying disease and viral infection CARs have not demonstrated the same high response rates to solid tumor antigens as have been seen against CD19-positive homological malignancies
CAR T	Serious immune toxicity (CRS) or neurotoxicity  Standard-of-care (steroids) and/or cytokine receptor antagonists, such as tocilizumab, can be ineffective; long ICU stay, relapse of underlying disease, infections and death  Other safety approaches* have slow onset of action or have safety issues of their own	Small number of validated tumor-specific antigens that can be targeted  For certain antigen targets, severe toxicity from treatment prevents sufficient therapeutic window for clinical benefit
TCR	High risk of off-target or off-organ toxicities	Human clinical data still early

\* See discussion of other approaches below under "Our Proprietary Switch Technologies - CaspaCIDE"

## 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

5

---

Table of Contents

infusion of a small molecule, rimiducid, instead of by natural upstream signals. Our current 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 apoptosis, and the “activation switch” is designed to lead to proliferation and/or activation and/or persistence 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 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-9i: Signaling Molecule for Apoptosis.** Caspase-9, or iCaspase is the initiating enzyme in the apoptosis pathway. When activated, caspase starts a signaling cascade, including the activation of caspase-3, which ultimately leads to apoptosis, a non-inflammatory process of cell elimination.

**iMC: 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.

#### 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 two most advanced switch technologies.

	CaspaCIDE	GoCAR-T
Cell Type	Donor T cells (HSCT) or patient T cells (TCRs)	Patient T cells
Proprietary Components	iCaspase - safety switch	iMC co-stimulation switch
Applications	HSCT and TCR therapy	CAR T therapy
Potential Safety Benefit	Modulation of effect with rimiducid triggers T cell apoptosis	Modulation of effect with rimiducid triggers T-cell activation & proliferation
Potential Efficacy Benefit	Widens therapeutic window for maximum benefit from treatment	Widens therapeutic window; iMC may enhance T cell activity
Product Candidates	BPX-501 and BPX-701	BPX-601

#### CaspaCIDE

CaspaCIDE is our CID based 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 iCaspase, an enzyme that is part of the apoptotic, cell death pathway. Infusion of rimiducid is designed to trigger activation of this domain of iCaspase, 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 provided after allogeneic HSCT, and to our TCR product candidate, BPX-701. We believe that CaspaCIDE is the optimal cell therapy safety switch technology described to-date. The only other widely reported clinically validated approach 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 nonclinical studies have demonstrated CaspaCIDE's potential benefits relative to HSV-tk, including 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 activity beginning as soon as 30 minutes after

## Table of Contents

administration of the activating drug, rimiducid. Lastly, rimiducid is bio-inert in the absence of cells containing a CID-based switch, and has no other clinical use. In contrast, ganciclovir has side effects, and physicians are reluctant to lose the ability to use it to treat herpes virus family infections in patients treated with HSV-tk-containing cells. Other cell elimination approaches described in the literature include gene modification of cells to express truncated epidermal growth factor receptor or codon-optimized CD20. Administration of the monoclonal antibodies cetuximab or rituximab, respectively, is intended to trigger complement-mediated cytotoxicity, or CMC, or 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 a CMC/ADCC-mediated mechanism may add to complications in patients already in an inflammatory crisis, such as seen with serious CRS, 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 have selectively entered into agreements with renowned cancer research centers with expertise in cellular immunotherapy to allow the use of our CaspaCIDE safety switch with the collaborators' CAR T product candidates. While we are not the sponsor of these clinical trials, we believe that they may facilitate the adoption of CaspaCIDE in the CAR T cell setting and provide opportunities for license arrangements of our technology in the future.

### 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 CAR on the surface 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, ultimately comprising 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 placing our proprietary co-stimulatory domain MC under

rimiducid control. 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 were found to be only fully activated when the GoCAR-T cells were exposed to both their target PSCA-expressing human pancreatic cancer cells and rimiducid. In further in vivo studies of GoCAR-T technology, target antigen PSCA-expressing HPAC human pancreatic tumors, which were established in immune-deficient mice, were eliminated by administration of GoCAR-T cells targeting PSCA along with weekly rimiducid administration. 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.

Our Product Candidates

BPX-501: Adjunct T Cell Therapy for Allogeneic Hematopoietic Stem Cell Transplantation

Our lead product candidate, BPX-501, is an adjunct T cell therapy administered after allogeneic HSCT using genetically modified donor T cells incorporating our CaspaCIDE safety switch. BPX-501, in combination with rimiducid, was recently granted orphan drug designation by the FDA for the treatment of immunodeficiency and GvHD following allogeneic HSCT, and is currently being evaluated in multiple Phase 1/2 clinical trials in adults and pediatric patients with leukemias, lymphomas and genetic blood diseases in the U.S. and Europe. We believe that BPX-501 could enable physicians to maximize the benefits of T cell therapy for allogeneic HSCT, such as immune system reconstitution, prevention or treatment of relapse of underlying disease and improvement in stem cell

## Table of Contents

engraftment, while mitigating some of the safety issues associated with a stem cell transplant. We reported initial top-line data from ongoing clinical trials in the HSCT setting in December 2016 at 58th Annual Meeting of the American Society of Hematology.

The goal of our BPX-501 clinical program is to provide better overall transplant outcomes-lower rates of infection and faster immune recovery-than one would generally expect from an alternative allogeneic transplant procedure. We are currently conducting multiple Phase 1/2 clinical trials of BPX-501 in the U.S. and Europe. In November 2014, we initiated BP-004, a Phase 1/2 clinical trial in children with leukemias, lymphomas, or orphan inherited blood disorders, such as severe combined immunodeficiency, Wiskott-Aldrich Syndrome and beta thalassemia, all chronic life-long disorders for which HSCT is curative. The trial is being conducted in both European and U.S. pediatric transplant centers. The clinical trial is evaluating whether BPX-501 T cells from a haploidentical donor, typically the child's mother or father, administered following a T-depleted HSCT, are safe and can enhance immune reconstitution. Additional ongoing clinical studies include BP-001, BP-005 and BP-008 in adults in which BPX-501 is administered after initial allogeneic HSCT for hematological cancers, and BP-003, a single site clinical trial in children with orphan inherited blood disorders in which BPX-501 is administered after initial allogeneic HSCT. In addition, we are planning to initiate additional Phase 1/2 clinical trials in the U.S. and Europe, as part of our strategy to pursue global regulatory approvals and expand the potential addressable patient population for BPX-501.

In July 2016, the intellectual property for BPX-501 was strengthened with a U.S. method of use patent issued to Baylor College of Medicine, or Baylor. The patent, licensed exclusively to us, is scheduled to expire in 2031. During 2016, we discussed with the EMA clinical and regulatory plans to support the filing of MAAs for BPX-501 and rimiducid in Europe, initially for pediatric patients with certain orphan inherited blood disorders or treatment-refractory hematological cancers. Based on regulatory discussions, we believe that data from the European arm of our BP-004 trial, expanded to enroll additional patients, could form the basis of MAAs for BPX-501 and rimiducid. In addition, the CHMP has agreed that review and approval under "exceptional circumstances" may be suitable, recognizing that a randomized trial may not be feasible in the pediatric setting. Exceptional circumstances may be granted for medicines that treat very rare diseases, or where controlled studies are impractical or not consistent with accepted principles of medical ethics. In place of a randomized trial, we intend to collect data from a concurrent observational study of allogeneic HSCT outcomes in the pediatric setting, in a total of approximately 120 patients, and include both retrospective patients and up to 40 prospective patients and up to 40 prospective patients, with a primary endpoint of event-free survival (with events defined as death, Grade 3-4 acute GvHD, chronic GvHD, and Grade 3-4 infection) at six months and expect to provide updates in the first half of 2017.

We have discussions ongoing with the FDA regarding the regulatory path to approval in the U.S. and expect to provide an update in the first half of 2017.

In addition to BPX-501, our clinical stage and preclinical product candidates, which are designed to overcome the current limitations of CAR T and TCR therapies, include the following:

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

We are developing BPX-601, a GoCAR-T product candidate containing Bellicum's proprietary iMC activation switch, for the treatment of solid tumors expressing PSCA. PSCA is a cancer antigen expressed in many malignancies, including prostate, pancreatic, bladder, esophagus, and gastric cancers. Preclinical data shows enhanced T-cell proliferation, persistence and in vivo anti-tumor activity compared to traditional CAR T therapies.

The initial planned indication for BPX-601 development is non-resectable pancreatic cancer. The BPX-601 initial Phase 1 protocol and related documents were reviewed by the National Institutes of Health, or NIH, Recombinant DNA Advisory Committee, or RAC, in March 2016. Subsequently, we filed an Investigational New Drug Application, or IND, for BPX-601, and a Phase 1 clinical trial in patients with non-resectable pancreatic cancer is now underway at the Baylor Sammons Cancer Center in Dallas, Texas.

In December, 2015 we entered into a license agreement with Agensys, Inc., or Agensys, an affiliate of Astellas Pharma Inc., under which we were granted an exclusive worldwide license for rights to PSCA and related antibodies.

### **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, or Leiden. BPX-701 is designed to target malignant cells expressing PRAME. As

initially reported in Clinical Cancer Research in 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. 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, BPX-701 cells containing the CaspaCIDE safety switch, have demonstrated complete elimination in response to the administration of rimiducid.



## Table of Contents

Planned indications for initial BPX-701 clinical development are refractory or relapsed AML and MDS, with an additional study planned for metastatic uveal melanoma. Each of these are orphan indications where PRAME is highly expressed and for which current treatment options are limited. The initial BPX-701 Phase 1 protocol and related documents were reviewed by the RAC in March 2016. Subsequently, we filed an IND for BPX-701, and we have initiated a Phase 1 clinical trial in patients with relapsed or refractory myeloid neoplasms at the Oregon Health & Science University Hospital in Portland, Oregon.

### Manufacturing, Processing and Delivering to Patients

We have developed an efficient and scalable process to manufacture genetically modified T cells of high quality. We have been working with third-party contract manufacturers in both Europe and the U.S. to produce BPX-501 for our clinical trials. We have leased an additional 30,400 square feet of space in our headquarters building in Houston, Texas and have construction ongoing to build out this space to facilitate in house manufacturing for the planned U.S. clinical and early commercial requirements for BPX-501, and the clinical supply needs of our other product candidates. This site has been designed and is being constructed to satisfy both U.S. and European regulatory requirements. Our current plan is to rely primarily on contract manufacturers for our European needs and have our U.S. facility qualified and available as a back-up site. We are leveraging the processes, as we have developed for BPX-501, as well as our resources, capabilities and expertise for the manufacture of our CAR T and TCR product candidates.

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 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 sufficient 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. The vector production is performed at multiple third-party supplier facility under good manufacturing practices, or GMPs, procedures and requirements.

**Genetically Modified T Cells.** We have agreements with reputable contract manufacturing organizations, or CMOs, with facilities in both the U.S. and Europe for processing and manufacturing our genetically modified T cells. We have started construction in the U.S. on a facility to allow the transition to in house manufacturing for the planned U.S. clinical and early commercial requirements for BPX-501, and the clinical supply needs of our other product candidates. 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 U.S. 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.

We are focused on continuously refining our overall cell therapy process, manufacturing, processing and delivery to patients to be more efficient. Our current process cycles for 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. We believe our patent estate, together with our efforts to develop and patent next generation technologies, provides us with a

## Table of Contents

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.

For example, 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 156 issued patents, 38 of which are in the U.S., and 60 pending patent applications, 17 of which are in the U.S., which we own or for which we have an exclusive, either in its entirety or within our field of use, commercial license as of February 28, 2017.

We have internally developed technology disclosed in two pending utility patent applications in the U.S. and thirteen pending foreign patent applications which relates to our GoCAR-T technology. If U.S. patents issue from the U.S. applications, the estimated expiration date of the last to expire patent is in 2035. If patents are issued in foreign jurisdictions, the anticipated expiration dates will be in 2035.

Pursuant to our licenses from Baylor, we have exclusive commercial rights to nine issued U.S. patents expiring in 2024 or later, four pending U.S. utility patent applications, nine issued foreign patents expiring in 2024 or later and 11 pending patent applications in foreign jurisdictions that relate to our GoCAR-T, BPX-501 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. If patents from the currently pending patent applications are issued in foreign jurisdictions, the estimated expiration dates range from 2024 to 2031.

Pursuant to our license agreement with ARIAD Pharmaceuticals, Inc., or ARIAD, as amended, we have exclusive commercial rights within our field of use to 22 patents, seven in the U.S. and 15 in foreign jurisdictions, which relate to dimerizer technology. The estimated expiration date of the last to expire U.S. patent is 2032. The estimated expiration date of the last to expire foreign patent is 2032.

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

Composition of matter patent coverage on rimiducid, the dimerization molecule AP1903, has expired. However, we believe that additional barriers to entry exist for a competitor attempting to use rimiducid. 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 biologic 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 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 U.S., 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, or the USPTO, 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

## Table of Contents

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 Collaboration and License Agreements

#### Co-Development and Co-Commercialization Agreement - Adaptimmune

In December 2016, we and Adaptimmune Therapeutics plc, or Adaptimmune entered into a Co-Development and Co-Commercialisation Agreement, or the Adaptimmune Agreement, in order to facilitate a staged collaboration to evaluate, develop and commercialize next generation T cell therapies.

Under the Adaptimmune Agreement, the parties agreed to evaluate our GoTCR technology, iMC co-stimulation, with Adaptimmune's affinity-optimized SPEAR® T cells for the potential to create enhanced TCR product candidates. Depending on results of the preclinical proof-of-concept phase, the parties expect to progress to a two-target co-development and co-commercialization phase. To the extent necessary, and in furtherance of the parties' proof-of-concept and co-development efforts, the parties granted each other a royalty-free, non-transferable, non-exclusive license covering their respective technologies for purposes of facilitating such proof-of-concept and co-development efforts. In addition, as to covered therapies developed under the Adaptimmune Agreement, the parties granted each other a reciprocal exclusive license for the commercialization of such therapies.

With respect to any joint commercialization of a covered therapy, the parties agreed to negotiate in good faith the commercially reasonable terms of a co-commercialization agreement. The parties also agreed that any such agreement shall provide for, among other things, equal sharing of the costs of any such joint commercialization and the calculation of profit shares as set forth in the Adaptimmune Agreement.

The Adaptimmune Agreement will expire on a country-by-country basis once the parties cease commercialization of the T cell therapies covered by the Adaptimmune Agreement, unless earlier terminated by either party for material breach, non-performance or cessation of development, bankruptcy/insolvency, or failure to progress to co-development phase.

#### Collaboration Agreement - OPBG

In October 2016, we and Ospedale Pediatrico Bambino Gesù, or OPBG, entered into a collaboration agreement, or the OPBG Agreement, pursuant to which we and OPBG agreed to collaborate on research projects and early stage clinical trials for the design and development of various T cell immunotherapies, or the OPBG Research.

As consideration for OPBG's performance of the OPBG Research and grant of certain licenses to us, we agreed to fund an aggregate of up to \$4.4 million in project costs payable to OPBG or certain third party service providers, as applicable, over the term of the OPBG Research, estimated to be four years. With respect to any inventions arising from the OPBG Research, OPBG agreed to grant us an exclusive license to any such inventions, the terms of which would be set forth in a separate agreement. In addition, OPBG granted us paid-up, worldwide co-exclusive licenses for non-commercial development of OPBG's CD19 and CAR.GD2 CAR T technologies, as well as paid-up, worldwide exclusive licenses to commercialize OPBG's CD19 and CAR.GD2 CAR T technologies, each to be governed by a separate agreement.

The initial term of the OPBG Agreement expires on June 30, 2017, unless the parties agree to an extension. Either party may terminate the OPBG Agreement upon written notice delivered 30 days in advance if the OPBG Research fails and such failure cannot be remedied within 60 days of such notice. We may terminate the OPBG Agreement at

any time upon providing OPBG with written notice 60 days in advance.

Collaboration Agreement - Leiden

In May 2016, we and Academisch Ziekenhuis Leiden, also acting under the name Leiden University Medical Centre, or Leiden, entered into a research collaboration agreement, or the Leiden Agreement, pursuant to which we will provide Leiden with financial support for research to discover and validate high-affinity TCR product candidates targeting several cancer-associated antigens, or the Research.

## Table of Contents

As consideration for Leiden's performance of the Research, we agreed to pay Leiden an aggregate of EUR 2,547,415 in quarterly installments during the three-year term of the Research. With respect to any inventions arising from the Research that are relevant to or useful for any high affinity TCR that is studied in the Research, Leiden granted us an exclusive option to obtain an exclusive, worldwide license to practice and exploit such inventions. The parties agreed to negotiate in good faith the commercially reasonable terms of each such license agreement entered into between the parties, based on terms similar to those set forth in the previously executed license agreement between the parties and those specified in the Leiden Agreement.

The Research will be conducted during a three-year term, after which the Leiden Agreement will expire. We and Leiden have agreed to negotiate in good faith a potential extension of such term, dependent on Leiden's progress in the performance of the Research. Either party may terminate the Leiden Agreement upon a material breach by the other party that remains uncured following 30 days after the date of written notice of such breach. Leiden may terminate the Leiden Agreement in the event of a failure by us to pay any amounts due under the Leiden Agreement that remains uncured on the date that is 30 days after written notice of such failure.

### License Agreement - Agensys

In December 2015, we and Agensys, entered into a license agreement, or the Agensys Agreement, pursuant to which (i) Agensys granted us, within the field of cell and gene therapy of diseases in humans, an exclusive, worldwide license and sublicense to its patent rights directed to PSCA and related antibodies, and (ii) we granted Agensys a non-exclusive, fully paid license to our patents directed to inventions that were made by us in the course of developing our licensed products, solely for use with Agensys therapeutic products containing a soluble antibody that binds to PSCA or, to the extent not based upon our other proprietary technology, to non-therapeutic applications of antibodies not used within the field.

As consideration for the rights granted to us under the Agensys Agreement, we agreed to pay to Agensys a non-refundable upfront fee of \$3.0 million. We are also required to make aggregate milestone payments to Agensys of up to (i) \$5.0 million upon the first achievement of certain specified clinical milestones for its licensed products, (ii) \$50.0 million upon the achievement of certain specified clinical milestones for each licensed product, and (iii) \$75.0 million upon the achievement of certain sales milestones for each licensed product. The Agensys Agreement additionally provides that we will pay to Agensys a royalty percentage that ranges from the mid to high single digits based on the level of annual net sales of licensed products by us, our affiliates or permitted sublicensees. The royalty payments are subject to reduction under specified circumstances.

Under the Agensys Agreement, Agensys also was granted the option to obtain an exclusive license, on a product-by-product basis, from us to commercialize in Japan each licensed product developed under the Agensys Agreement that has completed a phase 2 clinical trial. As to each such licensed product, if Agensys or its affiliate, Astellas Pharma, Inc., exercises the option, the Agensys Agreement provides that we will be paid an option exercise fee of \$5.0 million. In addition, the Agensys Agreement provides that we will be paid a royalty that ranges from the mid to high single digits based on the level of annual net sales in Japan of each such licensed product. If the option is exercised, the aggregate milestone payments payable by us to Agensys, described above with respect to each licensed product, would be reduced by up to an aggregate of \$65.0 million upon the achievement of certain specified clinical and sales milestones.

The Agensys Agreement will terminate upon the expiration of the last royalty term for the products covered by the Agensys Agreement, which is the earlier of (i) the date of expiration or abandonment of the last valid claim within the licensed patent rights covering any licensed products under the Agensys Agreement, (ii) the expiration of regulatory exclusivity as to a licensed product, and (iii) 10 years after the first commercial sale of a licensed product. Either party may terminate the Agensys Agreement upon a material breach by the other party that remains uncured following 60 days after the date of written notice of such breach (or 30 days if such material breach is related to failure to make payment of amounts due under the Agensys Agreement) or upon certain insolvency events. In addition, Agensys may terminate the Agensys Agreement immediately upon written notice to us if we or any of our affiliates or permitted sublicensees commence an interference proceeding or challenge the validity or enforceability of any of Agensys' patent rights.

License Agreement - BioVec

In June 2015, we and BioVec Pharma, Inc., or BioVec, entered into a license agreement, or the BioVec Agreement, pursuant to which BioVec agreed to supply us with certain proprietary cell lines and granted us a non-exclusive, worldwide license to certain of its patent rights and related know-how related to such proprietary cell lines.

As consideration for the products supplied and rights granted to us under the BioVec Agreement, we agreed to pay to BioVec an upfront fee of \$100,000 within ten business days of the effective date of the BioVec Agreement and a fee of \$300,000 within ten business days of its receipt of the first release of GMP lot of the products licensed under the BioVec Agreement. In addition, we agreed to pay to BioVec an annual fee of \$150,000, commencing 30 days following the first filing of an IND, or its foreign equivalent, for a product covered by the license; with such annual fees being creditable against any royalties payable by us to BioVec under the BioVec Agreement. We also are required to make a \$250,000 milestone payment to BioVec for each of the first three licensed products to enter



## Table of Contents

into a clinical phase trial and one-time milestone payments of \$2.0 million upon receipt of a registration granted by the FDA or EMA on each of our first three licensed products. The BioVec Agreement additionally provides that we will pay to BioVec a royalty in the low single digits on net sales of products covered by the BioVec Agreement. We may also grant sublicenses under the licensed patent rights and know-how to third parties for limited purposes related to the use, sale and other exploitation of the products licensed under the BioVec Agreement. The BioVec Agreement will continue until terminated. The BioVec Agreement may be terminated by us, in our sole discretion, at any time upon 90 days written notice to BioVec. Either party may terminate the BioVec Agreement in the event of a breach by the other party of any material provision of the BioVec Agreement that remains uncured on the date that is 60 days after written notice of such failure or upon certain insolvency events that remain uncured following the date that is 30 days after the date of written notice to a party regarding such insolvency event.

### License Agreement - Leiden

In April 2015, we and Leiden, entered into a license agreement, or the 2015 Leiden Agreement, pursuant to which Leiden granted to us an exclusive, worldwide license to its patent rights covering high affinity T-cell receptors targeting PRAME, and POU2AF1 epitopes. The license granted under the 2015 Leiden Agreement is subject to certain restrictions and to Leiden's retained right to use the licensed patents solely for academic research and teaching purposes, including research collaborations by Leiden with academic, non-profit research third parties; provided that Leiden provides 30 days advance written notice to us of such academic research collaborations.

As consideration for the rights granted to us under the 2015 Leiden Agreement, we agreed to pay to Leiden an aggregate of EUR 75,000 in upfront fees within 30 days of the effective date of the 2015 Leiden Agreement. In addition, we agreed to pay to Leiden, beginning on the eighth anniversary of the effective date of the 2015 Leiden Agreement, annual minimum royalty payments of EUR 30,000. We are also required to make milestone payments to Leiden of up to an aggregate of EUR 1,025,000 for each of the first licensed product that is specific to PRAME and to POU2AF1. The 2015 Leiden Agreement additionally provides that we will pay to Leiden a royalty in the low single digits on net sales of products covered by the 2015 Leiden Agreement. If we enter into a sublicensing agreement with a third party related to a product covered by the Leiden Agreement, we have agreed to pay Leiden a percentage ranging in the low double digits on all non-royalty income received from sublicensing revenue directly attributable to the sublicense, dependent on whether we are in phase 1/2, phase 2 or phase 3 at the time that we enter into any such sublicensing agreement.

Under the 2015 Leiden Agreement, we and Leiden entered into a sponsored research agreement, pursuant to which we are required to pay Leiden up to EUR 300,000 over a three-year period during the term of the sponsored research agreement. The 2015 Leiden Agreement will expire upon the expiration of the last patent included in the licensed patent rights. The 2015 Leiden Agreement may be terminated earlier upon mutual written agreement between us and Leiden, and at any time by us upon six months written notice to Leiden. Leiden may terminate the 2015 Leiden Agreement in the event of a failure by us to pay any amounts due under the 2015 Leiden Agreement that remains uncured on the date that is 30 days after written notice of such failure. Either party may terminate the 2015 Leiden Agreement upon a material breach by the other party that remains uncured following 30 days after the date of written notice of such breach or upon certain insolvency events that remain uncured following the date that is 45 days after the date of written notice to a party of such insolvency event.

### License Agreement - ARIAD Pharmaceuticals, Inc.

#### 2011 License Agreement

In March 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 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 original license from ARIAD, 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 original license agreement. In addition, we paid ARIAD a license fee of \$250,000 in connection with the restated license 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.

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., an ARIAD affiliate that merged into ARIAD, and the academic institution from with ARIAD

## Table of Contents

obtained its license to the underlying technology. While we are not required to pay royalties or fees to such academic institution, no sublicensee 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 \$15.0 million and we issued a promissory note to ARIAD for a principal amount of \$35.0 million 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 \$15.0 million 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 \$35.0 million, which included an additional payment of \$20.0 million 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 were terminated.

### License Agreements - 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 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 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.

## Table of Contents

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. Bellicum is 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, Bellicum is 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.

### 2016 Baylor License Agreements

In March 2016, we and Baylor entered into two additional license agreements pursuant to which we obtained exclusive rights to technologies and patent rights owned by Baylor. We paid Baylor a non-refundable license fee of \$100,000, and could incur additional payments upon the achievement of certain milestone events as set forth in the agreements. If we are successful in developing any of the licensed technologies under either agreement, resulting sales would be subject to a royalty payment in the low single digits.

### Grant Agreement

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

In July 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 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 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

## Table of Contents

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.

In November 2016, we announced that the Company received notice of a product development award totaling approximately \$16.9 million from CPRIT. Assuming successful contract negotiations and execution, the CPRIT award would fund a portion of a three-year global clinical program comprising clinical trials for adult and pediatric patients with high-risk and intermediate-risk AML. The proposed studies are designed to evaluate the benefit of BPX-501 and rimiducid in the context of in vivo and ex vivo T cell depleted haploidentical HSCT. The CPRIT oversight committee met in February 2017 and agreed to move forward with the proposed terms of the grant agreement. We are currently in the process of completing a new contract with CPRIT and expect to begin a clinical development program supported by the CPRIT funding in the second half of 2017.

### 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 is designed to provide improved outcomes through enhanced time to reconstitution of the immune system and address the safety risks of GvHD and susceptibility to infections. 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-601 and BPX-701 based on our GoCAR-T and CaspaCIDE technologies may compete with product candidates from a number of companies that are currently focused on this therapeutic modality, including Adaptimmune, bluebird bio, Inc., Celgene Corporation, Cellectis SA, Cell Medica Limited, GlaxoSmithKline plc, Intrexon Corporation, Immune Design Corp., Juno Therapeutics, Inc., Kiadis Pharma B.V., Kite Pharma, Inc., Lion Biotechnologies, Inc., Medigene AG, MolMed S.p.A., Novartis AG, Pfizer Inc., Unum Therapeutics, Precision Biosciences, Inc. and Ziopharm Oncology.

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 that product.



## Table of Contents

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 U.S., 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 the current good manufacturing practice, or 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 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, rimiducid. In general, biologics such as our engineered cells are regulated through the FDA’s Center for Biologics Evaluation and Research, or CBER, while synthetic drugs are regulated through the FDA’s Center for Drug Evaluation and Research. When the 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. The cellular component of our combination contributes the primary mode of action and, as a result, the FDA will regulate our investigational products as biologics, through CBER.

Government authorities in the U.S., at the federal, state and local levels, 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 U.S. 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 U.S., 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 U.S., 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. The 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 U.S. 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;
- 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;

## Table of Contents

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. Gene therapy clinical trials conducted at institutions that receive funding for recombinant DNA research from the NIH, are also subject to review by the RAC. Although the FDA decides whether individual gene therapy protocols may proceed, the RAC review process can delay the initiation of a clinical trial, even if the FDA has reviewed the trial design and details and approved its initiation. Conversely, the FDA can put an IND on clinical hold even if the RAC has provided a favorable review of the drug.

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.

## Table of Contents

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. This is a relatively new and expanding area of novel therapeutic interventions, and therefore there is uncertainty 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, and the eventual quality of data to be generated in these clinical trials for 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, and accordingly we disclose information on our clinical trials on [www.clintrials.gov](http://www.clintrials.gov). 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. The 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

## Table of Contents

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, the 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 U.S., or more than 200,000 individuals in the U.S. and for which there is no reasonable expectation that the cost of developing and making available in the U.S. a drug or biologic for this type of disease or condition will be recovered from sales in the U.S. 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 the 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.



## Table of Contents

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 U.S. 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.

On February 22, 2016, we announced that the FDA granted orphan drug designation for the combination of BPX-501 genetically modified T cells and activator agent rimiducid as "replacement T-cell therapy for the treatment of immunodeficiency and graft versus host disease(GvHD) after allogeneic hematopoietic stem cell transplant."

BPX-501 is an adjunct T-cell therapy incorporating our proprietary CaspaCIDE safety switch.

### 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 efficacy. 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, the 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-501, BPX-601 and BPX-701. 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

## Table of Contents

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. 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 USPTO, 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 U.S., 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, or HHS, such as 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 Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, collectively, the Affordable Care Act, and similar state laws, each as amended.

## Table of Contents

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 either the referral of an individual for, or the 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. The Anti-Kickback Statute may be violated if only one purpose of the remuneration is to induce referrals. 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 law 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, that is, 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 their 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, 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 require that certain manufacturers and group purchasing organizations 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 Drug Quality and Security Act.

## Table of Contents

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, individual imprisonment, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, 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 U.S. 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 U.S., 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 U.S. 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 was intended to broaden access to health insurance, improve quality, and reduce or constrain the growth of healthcare spending among other health policy reforms. The Affordable Care Act has substantially changed healthcare financing and delivery by both governmental and private insurers, and continues to significantly



## Table of Contents

impact the pharmaceutical and biotechnology industry. The Affordable Care Act has changed existing government healthcare programs and resulted 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;

- 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.

Since its enactment there have been judicial and Congressional challenges to certain aspects of the Affordable Care Act. In January, Congress voted to adopt a budget resolution for fiscal year 2017, or the Budget Resolution, that authorizes the implementation of legislation that would repeal portions of the Affordable Care Act. The Budget Resolution is not a law, however, it is widely viewed as the first step toward the passage of repeal legislation. Further, on January 20, 2017, President Trump signed an Executive Order directing federal agencies with authorities and responsibilities under the Affordable Care Act to waive, defer, grant exemptions from, or delay the implementation of any provision of the Affordable Care Act that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. Congress also could consider subsequent legislation to replace elements of the Affordable Care Act that are repealed.

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, President Obama 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, due to subsequent legislative amendments will stay in effect through 2025 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. Further, there has been heightened governmental scrutiny recently over the manner in which manufacturers set prices for their marketed products. For example, there have been several recent Congressional inquiries and proposed bills designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the cost of drugs under Medicare, and reform government program reimbursement methodologies for drug products. 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 U.S. to comply with accounting provisions requiring the company to maintain

## Table of Contents

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 U.S., 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 U.S. 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 U.S. 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, 2016, we had 110 employees, all of whom were full-time, 95 of whom were engaged in research and development activities and 15 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 IPO 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, or (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.

## Table of Contents

### 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, have no products approved for commercial sale 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 fiscal years ended December 31, 2016 and 2015, we reported a net loss of \$69.2 million and \$48.5 million, respectively.

As of December 31, 2016, we had an accumulated deficit of \$230.7 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 and as we plan for the potential commercial launch of our lead product candidate, BPX-501.

Even if we succeed in commercializing BPX-501 or other 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 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, including BPX-501. 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 BPX-501 and our other current product candidates, as well as the product candidates that are being developed by our partners and licensees;
- seeking and obtaining marketing approvals for BPX-501 and any other product candidates that successfully complete clinical trials, if any;
- launching and commercializing BPX-501 and other product candidates for which we obtain marketing approval, if any, 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



## Table of Contents

trials in addition to those that we currently anticipate for BPX-501 and our other product candidates, or if there are any delays in our or our partners completing clinical trials or the development of any of our product candidates, including BPX-501. If one or more of the product candidates that we independently develop is approved for commercial sale, we expect to incur significant costs associated with commercializing any 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 successfully commercialize and launch BPX-501 and 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 and the success of BPX-501.

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;
- establishing sales and marketing capabilities upon obtaining any regulatory approval to gain market acceptance of a novel therapy; and
- the availability of coverage and adequate reimbursement from third-party payors for our novel and personalized 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.

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 still 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 expect to 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, regulatory issues or other problems, our development plans and business for our other product candidates would be significantly harmed.





## Table of Contents

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, including for BPX-501, 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 larger-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-601 and 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, the FDA or foreign regulatory authorities may not 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 our clinical trials, including for BPX-501, 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 and GoCAR-T 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.

## Table of Contents

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 could require us to perform additional clinical trials before approving our marketing applications. It is possible that, upon inspection, such regulatory authorities could determine that any of our clinical trials fail to comply with the cGCP regulations. In addition, our clinical trials must be conducted with biologic product produced under 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.

## Table of Contents

In particular, some of our clinical trials will look to enroll patients with characteristics which are found in a very small population, for example, 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 companies' 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 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

## Table of Contents

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.

BPX-501 and rimiducid have received orphan drug designation, but we may be unable to maintain or receive the benefits associated with orphan drug status, including market exclusivity.

The FDA or European Commission 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 that jurisdiction a drug or biologic for a disease or condition will be recovered from sales in that jurisdiction for that drug or biologic. If a product that has orphan drug designation subsequently receives the first FDA or European Commission approval for the disease for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA or European Commission may not approve any other applications, including a full authorization to market the same biologic for the same indication for seven years in the U.S. and for 10 years in Europe, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity.

The European Commission has granted orphan drug designations to BPX-501 for treatment in HSCT, and for activator agent rimiducid for the treatment of GvHD. Additionally, BPX-501 and rimiducid have received orphan drug designation from the FDA, as a combination replacement T-cell therapy for the treatment of immunodeficiency and GvHD after allogeneic HSCT. However, in each case exclusive marketing rights may be limited if we seek approval for an indication broader than the orphan designated indication and may be lost if the European Commission or FDA, as applicable, later determines that the request for designation was materially defective or if we are unable to assure the availability of sufficient quantities of the product to meet the needs of patients with the rare disease or condition. Although the respective designations may provide seven years of market exclusivity in the U.S. and 10 years of market exclusivity in Europe, the designations are subject to certain limited exceptions. Therefore, even though we have obtained orphan drug designation for certain indications, we may be unable to obtain orphan drug designation for our future product candidates and we may not be the first to obtain marketing approval for any particular orphan indication.

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, 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. 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.”



## Table of Contents

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 President and Chief Executive Officer, our Chief Financial Officer and Treasurer and our Chief Operating Officer and Executive Vice President of Clinical Development. 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 March 1, 2017, we had 115 employees. As our development and commercialization plans and strategies develop for the potential launch of BPX-501, and as we continue our transition into operating as a public company, we expect to need additional managerial, operational, sales, marketing, financial and other personnel. Future growth imposes 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 these 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. The services of independent organizations, advisors and consultants may not continue to be available to us on a timely basis when needed, and we may not be able to 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. We may not 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, including BPX-501, and, accordingly, may not achieve our research, development and commercialization goals.

In addition to expanding our organization, we are increasing the size of our facility and building out our development and manufacturing capabilities, which requires 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.

## Table of Contents

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 BPX-501 and our other product candidates.

As of December 31, 2016, we had cash and cash equivalents of approximately \$33.1 million and total investments in marketable securities of \$70.6 million. We believe that cash and cash equivalents and investments in marketable securities, or a total of \$103.7 million, will be sufficient to fund our operations through the first quarter of 2018.

We maintain our cash, cash equivalents, and marketable securities with high quality, accredited financial institutions. These amounts at times may exceed federally insured limits. We have not experienced any credit losses in such accounts and do not believe we are exposed to significant risk on these funds. We have no off-balance sheet concentrations of credit risk, such as foreign currency exchange contracts, option contracts or other hedging arrangements.

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 expect to require additional capital for the further development and commercialization of our product candidates. 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.

The terms of our debt facility place restrictions on our operating and financial flexibility, and failure to comply with covenants or to satisfy certain conditions of the agreement governing the debt facility may result in acceleration of our repayment obligations and foreclosure on our pledged assets, which could significantly harm our liquidity, financial condition, operating results, business and prospects and cause the price of our common stock to decline.

In March 2016, we entered into a loan and security agreement with Hercules Capital, Inc., Hercules Technology II, L.P., and Hercules Technology III, L.P., or collectively, Hercules, that is secured by a lien covering substantially all of our assets, excluding intellectual property, but including proceeds from the sale, license, or disposition of our intellectual property under which we have borrowed \$30.0 million. The loan and security agreement governing the debt facility requires us to comply with a number of covenants (affirmative and negative), including restrictive covenants that limit our ability to: incur additional indebtedness; encumber the collateral securing the loan; acquire, own or make investments; repurchase or redeem any class of stock or other equity interest; declare or pay any cash dividend or make a cash distribution on any class of stock or other equity interest; transfer a material portion of our assets; acquire other businesses; and merge or consolidate with or into any other organization or otherwise suffer a change in control, in each case subject to exceptions. Our intellectual property also is subject to customary negative covenants. In addition, subject to limited exceptions, Hercules could declare an event of default upon the occurrence of any event that it interprets as having a material adverse effect upon our business, operations, properties, assets, or financial condition or upon our ability to perform or pay the secured obligations under the loan and security agreement or upon the collateral or Hercules' liens on the collateral under the agreement, thereby requiring us to repay the loan immediately, together with a prepayment charge of up to 2% of the then outstanding principal balance and an end-of-term charge of \$2.085 million. Although, in and of itself, the occurrence of adverse results or delays in any

clinical study or the denial, delay or limitation of approval of or taking of any other regulatory action by the FDA or another governmental entity will not constitute a material adverse effect under our loan and security agreement with Hercules, Hercules may determine that such an event together with contemporaneous events or circumstances constitutes a material adverse effect upon our business, operations, properties, assets, or financial condition or upon our ability to perform or pay the secured obligations under the loan and security agreement. If we default under the facility, Hercules may accelerate all of our repayment obligations and, if we are unable to access funds to meet those obligations or to renegotiate our agreement, Hercules could take control of our pledged assets and we could immediately cease operations. If we were to renegotiate our agreement under such circumstances, the terms may be significantly less favorable to us. If we were liquidated, Hercules' right to repayment would be senior to the rights of our stockholders to receive any proceeds from the

## Table of Contents

liquidation. Any declaration by Hercules of an event of default could significantly harm our liquidity, financial condition, operating results, business, and prospects and cause the price of our common stock to decline.

We may incur additional indebtedness in the future. The debt instruments governing such indebtedness may contain provisions that are as, or more, restrictive than the provisions governing our existing indebtedness under the loan and security agreement with Hercules. If we are unable to repay, refinance or restructure our indebtedness when payment is due, the lenders could proceed against the collateral or force us into bankruptcy or liquidation.

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, including through the sale of securities from our registration statement on Form S-3 filed with the U.S. Securities and Exchange Commission, or SEC, 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 the 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 Europe.

We do not currently own a European 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.

## Table of Contents

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 complete our own manufacturing facility for supply of U.S. clinical and/or commercial cell therapy product candidate requirements, but we may not be able to do so.

We have leased space and initiated work for the design and build out of manufacturing space at our headquarters building in Houston, Texas. 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 these 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 these 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 processes, there are timing and operational risks 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, we may not be able to establish or maintain such collaborative arrangements, or if we are able to do so, that they may not 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.



## Table of Contents

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 U.S. 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, including BPX-501, outside of the U.S. and, accordingly, 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 U.S.;
- potential liability under the FCPA 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 U.S.;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad;
- differing payor reimbursement regimes, governmental payors or patient self-pay systems and price controls; 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 and 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. It is possible that, following a strategic transaction or license, we may not 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,



Table of Contents

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 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, including BPX-501. 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 U.S. 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.

## Table of Contents

System outages, network disruptions and cyber-security threats could interrupt the operation of our business. We are dependent on the use of information technology systems for our operations. Outages, disruptions and threats could have an adverse impact on our ability to conduct operations. Cyber-security threats, such as malware, phishing and network attacks, are on the rise. These attacks can affect the availability of our information technology systems, including their data, as well as the confidentiality and integrity of these systems. A security breach poses a risk to confidential data, including but not limited to intellectual property and trade secrets resulting in financial, legal or reputational harm to us. Insider threats may exist if an individual authorized to access our technology systems improperly discloses sensitive data to unauthorized persons or the public. We also have outsourced elements of our operations, including elements of our information technology infrastructure, and thus manage several independent vendor relationships with third parties who may have access to our confidential information. Confidentiality agreements are in place for authorized users and third parties to support the prevention of confidential information being improperly disclosed. We have policies and procedures in place, including controls around the access and activity of authorized users, active system monitoring, back-up and recovery, information technology security and mandatory annual information technology security awareness training to assist in the prevention and mitigation of an outage, disruption or threat. In addition, we have invested in high availability, redundant technologies that will reduce the risk of an outage, disruption or threat. However, our efforts may not prevent an outage, disruption or threat that would materially adversely affect us. We also may not have sufficient liability insurance, either type or amount, to cover us against claims related to a cyber-security threat.

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 U.S. 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 U.S., 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 for, 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;

federal civil and criminal false claims laws and civil monetary penalties law, 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;

• 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,

## Table of Contents

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 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, 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 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 require certain manufacturers and group purchasing organizations to report annually 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, individual imprisonment, additional reporting requirements and/or oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, 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 U.S. will also 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 or cease 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;

- initiation of investigations by regulators;
- costs to defend the related litigation;
- a diversion of management's time and our resources;

40

---



## Table of Contents

- 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, with other coverage limits as appropriate for certain foreign jurisdictions. 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 initial IPO in December 2014 and our 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, 2016, we had gross federal income tax net operating loss, or NOL, carry forwards of \$142.2 million and federal research tax credits of \$4.3 million. The NOL carryforwards will expire beginning in 2025, if not utilized.

### 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;
- recruiting suitable patients to participate in a clinical trial;
- having patients complete a clinical trial or return for post-treatment follow-up;
- 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.

## Table of Contents

Gene therapy clinical trials conducted at institutions that receive funding for recombinant DNA research from the NIH, are also subject to review by the RAC. Although the FDA decides whether individual gene therapy protocols may proceed, the RAC review process can delay the initiation of a clinical trial, even if the FDA has reviewed the trial design and details and approved its initiation. Conversely, the FDA can put an IND on clinical hold even if the RAC has provided a favorable review of the drug. Also, before a clinical trial can begin at an NIH-funded institution, that institution's independent institutional review board, or IRB, and its Institutional Biosafety Committee must review the proposed clinical trial to assess the safety of the trial. In addition, adverse developments in clinical trials of gene therapy products conducted by others may cause the FDA or other regulatory bodies to change the requirements for approval of any of our product candidates.

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 a mismatched allogeneic (donor cells as opposed to the patient's own cells) HSCT. We have initiated dialogue with regulators in the U.S. to discuss our clinical trial design that could serve as the registration trial 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 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 US and European regulators 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 regulatory marketing 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 regulatory authorities 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;
- 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;

## Table of Contents

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 U.S. or elsewhere;

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.

Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and greater than, those in the U.S., 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

We may also submit marketing applications in other countries. Regulatory authorities in jurisdictions outside of the U.S. 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 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 U.S. 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

## Table of Contents

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. Many 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.

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.

## Table of Contents

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:

- 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 U.S., 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 U.S. 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 those 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 U.S. 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 Affordable Care Act was enacted in the U.S. The Affordable Care 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. Since its enactment there have been judicial and Congressional challenges to certain aspects of the Affordable Care Act, and we expect there will be additional challenges and amendments to it in the future. In January, Congress voted to adopt a budget resolution for fiscal year 2017, or the Budget Resolution, that authorizes the implementation of legislation that would repeal portions of the Affordable Care Act. The Budget Resolution is not a law, however, it is widely viewed as the first step toward the passage of repeal legislation. Further, on January 20, 2017, President Trump signed an Executive Order directing federal agencies with authorities and responsibilities under the Affordable Care Act to waive, defer, grant exemptions from, or delay the implementation of any provision of the Affordable Care Act that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. Congress also could consider subsequent legislation to replace elements of the Affordable Care Act that are repealed. We cannot predict how the Affordable Care Act, its possible repeal, or any legislation that may be proposed to replace the Affordable Care Act will impact our business.



Other legislative changes have been proposed and adopted in the U.S. since the Affordable Care 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 due to subsequent legislative amendments, will stay in effect through 2025 unless Congressional action is

## Table of Contents

taken. In January 2013, then 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. Further, there has been heightened governmental scrutiny recently over the manner in which manufacturers set prices for their marketed products. For example, there have been several recent Congressional inquiries and proposed bills designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the cost of drugs under Medicare, and reform government program reimbursement methodologies for drug products.

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, including BPX-501, 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 for BPX-501 and our other potential product candidates 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, for example, reimbursement for administration of our product candidates 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.

We are subject to certain U.S. and foreign anti-corruption, anti-money laundering, export control, sanctions, and other trade laws and regulations, or collectively, Trade Laws. We can face serious consequences for violations.

Among other matters, Trade Laws prohibit companies and their employees, agents, clinical research organizations, legal counsel, accountants, consultants, contractors, and other partners from authorizing, promising, offering, providing, soliciting, or receiving directly or indirectly, corrupt or improper payments or anything else of value to or from recipients in the public or private sector. Violations of Trade Laws can result in substantial criminal fines and civil penalties, imprisonment, the loss of trade privileges, debarment, tax reassessments, breach of contract and fraud litigation, reputational harm, and other consequences. We have direct or indirect interactions with officials and employees of government agencies or government-affiliated hospitals, universities, and other organizations. We engage third parties for clinical trials and/or to obtain necessary permits, licenses, patent registrations, and other regulatory approvals. We also expect our non-U.S. activities to increase in time. We can be held liable for the corrupt or other illegal activities of our personnel, agents, or partners, even if we do not explicitly authorize or have prior knowledge of such activities.



## Table of Contents

### 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, to certain genetic constructs and to certain methods for inducing apoptosis. 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 of Texas may have rights to commercialize the results of those clinical trials if it determines that we have failed, after notice and an opportunity to cure, to use diligent and commercially reasonable efforts to commercialize or otherwise bring to practical application the results of the funded clinical trials. We are also dependent on our license agreements with Agensys with respect to BPX 601, Leiden with respect to BPX 701 and BioVec with respect to making retrovirus for all of our programs. The termination of any of these licenses could have a material adverse effect on our business.

Any termination of these agreements, or other agreements to which we are a party 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.



## Table of Contents

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 U.S. 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 U.S. 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 U.S. 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 U.S. 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 U.S. and most other countries are confidential for a period of time after filing, it is possible that patent applications in our portfolio may not be the first filed patent applications related to our product candidates. Furthermore, for U.S. 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 U.S. 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.

Patent coverage on the dimerization molecule rimiducid, expired in February 2016. Therefore, any additional barriers to entry for competitors to use rimiducid 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 PHSA 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 twelve 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, it is possible that our trade secrets and other confidential proprietary information could be disclosed or that competitors may 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 U.S. As a result, we may encounter significant problems in protecting and defending our intellectual property both in the U.S. 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

## Table of Contents

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 parties 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. It is possible that any such license would not be available at all or 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.

For example, 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-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.

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.

Also, while we are aware there are other third party patents having claims that may be considered relevant to 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

## Table of Contents

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 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.

We may not 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 or other proceedings 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. It also is possible that a competitor we sue for patent infringement could countersue us for allegedly infringing one or more of their own patents or one or more patents they licensed from another entity. 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 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. It also is possible that third parties could institute a patent office post-grant proceeding against one or more of our patents, or one or more patents licensed to us, such as a post grant review proceeding, inter partes review proceeding or reexamination proceeding at the USPTO, or an opposition proceeding in a jurisdiction outside the U.S. An unfavorable outcome in a post-grant proceeding could result in a loss

of our patent rights. Litigation, interference proceedings or patent office post-grant 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 also 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 U.S.

## Table of Contents

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 U.S., 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 U.S. or abroad, even outside the context of litigation. Such mechanisms include re-examination, post grant review, and equivalent proceedings in foreign jurisdictions, for example, opposition proceedings. Any 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. 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 U.S. 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 U.S. 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 U.S. can be less extensive than those in the U.S. In addition, the laws of some foreign countries do not protect intellectual property to the same extent as federal and state laws in the U.S. Consequently, we may not be able to prevent third parties from practicing

## Table of Contents

our inventions in all countries outside the U.S., or from selling or importing products made using our inventions in and into the U.S. 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 U.S. 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.

### 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 December 2014 IPO, there was no public market for our common stock. The trading price of our common stock is likely to continue to be highly volatile and subject to wide fluctuations in response to various factors, some of which are beyond our control, including market conditions in general and a limited trading volume for our shares. 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 our ongoing or future clinical trials, including for BPX-501;
- 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;

## Table of Contents

our inability to maintain successful collaborations or to establish new 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. In addition, the terms of our loan and security agreement with Hercules restrict our ability to declare or pay any cash dividend or make a cash distribution on any class of stock or other equity interest. 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 February 28, 2017, our executive officers, directors and 10% stockholders beneficially owned approximately 31.4% of our outstanding voting shares. Therefore, these stockholders may have the ability to significantly influence us through this ownership



Table of Contents

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 the reduced reporting requirements applicable to emerging growth companies may make our common stock less attractive to investors.

We are an emerging growth company, as defined in the 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 IPO, December 23, 2014, 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 IPO, (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 requires the market value of our common stock that is held by non-affiliates to exceed \$700.0 million as of the prior June 30th, or (2) the date on which we have issued more than \$1.0 billion in non-convertible debt during the prior three-year period.

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 US GAAP, 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 our IPO on December 23, 2014. As a new public company, we will continue to 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 IPO. We intend to take advantage of this new legislation, but it is possible that we will 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.

## Table of Contents

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.

Certain holders of our outstanding shares of common stock, are entitled to rights with respect to the registration of their shares under the Securities Act of 1933, as amended, or Securities Act. Any sales of these shares by such stockholders could have a material adverse effect on the trading price of our common stock.

We register on Form S-8 all shares of common stock that are issuable under our 2014 Equity Incentive Plan, as amended, or the EIP. As a consequence, these shares can be freely sold in the public market upon issuance, subject to volume limitations applicable to affiliates.

Future sales and issuances of our common stock or rights to purchase common stock, including pursuant to our EIP and recently filed shelf registration statement, could result in additional dilution of the percentage ownership of our stockholders and could cause our stock price to fall.

We expect that significant additional capital will be needed in the future to continue our planned operations, including conducting clinical trials, commercialization efforts for BPX-501, 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, including pursuant to our shelf registration statement on Form S-3 that we filed with the SEC. If we sell common stock, convertible securities or other equity securities, investors may be materially diluted by subsequent sales. Any such sales may also result in material dilution to our existing stockholders, and new investors could gain rights, preferences and privileges senior to the existing holders of our common stock.

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

Our management has broad discretion in the application of the net proceeds from our December 2014 IPO. Because of the number and variability of factors that will determine our use of the net proceeds from our IPO, 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 IPO 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 IPO in ways that enhance stockholder value, we may fail to achieve 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;
- 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;

55

---

## Table of Contents

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 depends in part on the research and reports that securities or industry analysts publish about us or our business. In the event securities or industry analysts that cover us downgrade our stock or publish inaccurate or unfavorable research about our business, our stock price may decline. If one or more of these analysts ceases coverage of us 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

None.

### ITEM 2. Properties

We lease an aggregate of approximately 65,608 square feet of space in Houston, Texas, which consists of a 35,251 square foot facility for administrative and research and development activities under a lease that expires in January, 2020 with five, one-year lease renewal options, and a 30,357 square foot facility for in-house cell therapy manufacturing activities under a lease that expires in August 2026, with an option to renew for one additional period of five years. During 2016, the Company leased an aggregate of 3,540 additional square foot primarily for manufacturing and clean room space which is included in the manufacturing square footage above. 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.

Table of Contents

## 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 under the symbol "BLCM." Prior to such time, there was no public market for our common stock.

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 periods indicated:

	Price Range	
	High	Low
Year Ended December 31, 2014		
Fourth Quarter (commencing December 18, 2014)	\$27.38	\$18.20
Year Ended December 31, 2015		
First Quarter	\$33.63	\$19.73
Second Quarter	\$29.33	\$20.20
Third Quarter	\$21.71	\$13.66
Fourth Quarter	\$23.84	\$12.25
Year Ended December 31, 2016		
First Quarter	\$20.25	\$7.24
Second Quarter	\$13.75	\$8.61
Third Quarter	\$21.58	\$12.71
Fourth Quarter	\$23.11	\$13.50

## Holders of Record

As of February 28, 2017, there were approximately 41 stockholders of record of our common stock. Certain shares are held in "street" name and thus the actual 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. In addition, the terms of our loan and security agreement with Hercules restrict our ability to declare or pay any cash dividend or make a cash distribution on any class of stock or other equity interest. 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.

## 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.

## Stock 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 Composite Index (^IXIC), and the NASDAQ Biotechnology Index (^NBI). 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 SEC and are not intended to forecast or be indicative of possible future performance of our common stock.



Table of Contents

	Cumulative Total Return Date Ended								
	12/18/2013	3/31/2015	6/30/2015	9/30/2015	12/31/2015	3/31/2016	6/30/2016	9/30/2016	12/31/2016
	(Inception)								
Bellicum	\$100.00	\$ 94.34	\$ 86.60	\$ 59.16	\$ 82.53	\$ 38.07	\$ 52.77	\$ 81.03	\$ 55.46
Nasdaq Composite	\$100.00	\$ 111.41	\$ 119.70	\$ 98.16	\$ 109.66	\$ 84.46	\$ 83.42	\$ 93.76	\$ 85.88
Nasdaq Biotechnology	\$100.00	\$ 104.00	\$ 105.82	\$ 98.04	\$ 106.26	\$ 103.34	\$ 102.76	\$ 112.72	\$ 114.23

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

None.

## Use of Proceeds from Initial Public Offering

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. Since the effective date of our registration statement through the date of these financial statements, we have used approximately \$98.8 million of the proceeds to fund our operating activities, and the remainder is invested in cash and cash equivalent securities, or highly-liquid investment securities. See Notes 3 and 4 to the audited financial statements contained herein.



Table of Contents

## ITEM 6. Selected Financial Data

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

We derived the statements of operations data for the years ended December 31, 2016, 2015 and 2014 and the balance sheet data as of December 31, 2016 and 2015 from our audited financial statements included in this annual report. We derived the statements of operations data for the years ended December 31, 2013 and 2012 and the balance sheet data as of December 31, 2014, 2013 and 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.

	Year Ended December 31,				
	2016	2015	2014	2013	2012
	(in thousands, except share data)				
Statement of Operations:					
Grant revenues	\$388	\$282	\$1,780	\$1,941	\$1,470
Operating expenses:					
Research and development	51,263	33,561	12,071	7,899	6,156
License fees	580	3,184	—	—	—
ARIAD restructuring costs	—	—	43,212	—	—
General and administrative	16,925	12,672	4,335	1,964	1,583
Total operating expenses	68,768	49,417	59,618	9,863	7,739
Loss from operations	(68,380 )	(49,135 )	(57,838 )	(7,922 )	(6,269 )
Interest income	909	641	35	4	7
Interest expense	(1,760 )	(12 )	(1,791 )	(51 )	(1 )
Loss on disposal of assets	(10 )	(42 )	—	—	—
Change in fair value of warrant liability	—	—	(24,371 )	—	—
Net loss	\$(69,241 )	\$(48,548 )	\$(83,965 )	\$(7,969 )	\$(6,263 )
Preferred stock dividends	—	—	(1,432 )	(1,093 )	(757 )
Net loss attributable to common stockholders	\$(69,241 )	\$(48,548 )	\$(85,397 )	\$(9,062 )	\$(7,020 )
Basic and diluted net loss per share	\$(2.57 )	\$(1.84 )	\$(34.04 )	\$(5.05 )	\$(4.26 )
Weighted average common shares outstanding— basic and diluted	26,950,906	26,346,603	2,508,960	1,795,992	1,648,198

	As of December 31,				
	2016	2015	2014	2013	2012
	(in thousands)				
Balance Sheet Data:					
Cash, cash equivalents, restricted cash and investment securities	\$113,412	\$150,365	\$191,602	\$11,168	\$1,632
Working capital	90,497	89,445	189,586	9,963	256
Total assets	132,037	160,406	195,794	14,942	5,186
Capital lease obligation, net of current portion	141	118	—	—	—
Long-term debt, net of current portion	18,436	—	—	400	—
Convertible preferred stock	—	—	—	39,926	21,658
Accumulated deficit	(230,733 )	(161,492 )	(112,944 )	(28,979 )	(21,010 )
Total stockholders’ equity (deficit)	96,574	152,017	191,636	(28,152 )	(19,473 )



## Table of Contents

### 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 in "Item 8 - Financial Statements and Supplementary Data" 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 CID technology platform to engineer our product candidates with switch technologies that can 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. For additional information about our business, and candidate development programs, see the discussions contained within Item 1. Business in this Annual Report.

#### Recent Developments

On January 30, 2017, Thomas J. Farrell resigned from his position as a director and our President and Chief Executive Officer. In connection with Mr. Farrell's resignation, effective January 30, 2017, our board of directors appointed Richard A. Fair, 48, to serve as our President and Chief Executive Officer and, upon recommendation of the Nominating and Governance Committee of our Board, appointed Mr. Fair to our Board as a Class III director to hold office until the 2017 Annual Meeting of Stockholders.

Prior to joining Bellicum, Mr. Fair served as Senior Vice President, Therapeutic Head Oncology Global Product Strategy at Genentech, Inc., a private biotechnology company and subsidiary of Roche Holding AG. From April 2006 to January 2014, Mr. Fair held other positions at Genentech, including Vice President, Global Product Strategy Hematology & Signaling, from November 2012 through December 2013, and Vice President, Sales & Marketing, Oral Oncolytics, from May 2010 to November 2012. Prior to Genentech, Mr. Fair held positions at Johnson & Johnson, a public pharmaceutical and medical device company. Mr. Fair received his B.S. in computer science from the University of Michigan and his MBA, with a dual concentration in finance and management, from Columbia University.

On March 8, 2017, we borrowed an additional \$10.0 million under our existing debt facility with Hercules Capital, Inc., Hercules Technology II, L.P., and Hercules Technology III, L.P., or collectively, Hercules. We now have total outstanding principal under the loan agreement of approximately \$30.0 million and an aggregate end-of term charge of \$2.085 million. In addition, the interest only period was extended for another six months. See Notes 7 and 15 to the audited financial statements included herein.

In February 2017, we began the manufacturing of BPX-501 in our new manufacturing facility in Houston, Texas. We intend to manufacture for U.S. clinical trials of BPX-501 and our other product candidates and for initial commercial supply requirements for BPX-501 from our facility.

Also, in February 2017, we enrolled our first patient in the clinical trial of BPX-601, a GoCAR-T product candidate containing our proprietary iMC, inducible MyD88/CD40 activation switch, designed to treat solid tumors expressing PSMA.

On December 16, 2016, we entered into a Co-Development and Co-Commercialisation Agreement with Adaptimmune, or Adaptimmune, to evaluate, develop, and commercialize next-generation T-cell therapies. Under the

Adaptimmune Agreement, the parties will evaluate our GoTCR technology (inducible MyD88/CD40 co-stimulation, or iMC) with Adaptimmune's affinity-optimized SPEAR® T-cells for the potential to create enhanced TCR product candidates. Depending on results from the preclinical proof-of-concept phase, the parties expect to progress to a two-target co-development and co-commercialization phase.

On November 16, 2016, we received notice of a Product Development award totaling approximately \$16.9 million from CPRIT to support clinical studies of its lead product candidate BPX-501. Assuming successful contract negotiations and execution, the CPRIT award would fund a portion of a three-year global clinical program comprising clinical trials for adult and pediatric patients with high-risk and intermediate-risk acute myeloid leukemia. The proposed studies are designed to evaluate the benefit of BPX-501 and rimiducid in the context of in vivo and ex vivo T cell depleted haploidentical hematopoietic stem cell transplantation. The CPRIT oversight committee met in February 2017 and agreed to move forward with the proposed terms of the grant agreement.

## Table of Contents

On October 28, 2016, we entered into a collaboration agreement with OPBG, pursuant to which we and OPBG agreed to collaborate on research projects and early stage clinical trials for the design and development of various T cell immunotherapies. As consideration for OPBG's performance of the research under the agreement and grant of certain licenses to us, we agreed to fund an aggregate of up to \$4.7 million in project costs payable to OPBG or certain third party service providers, as applicable, over the term of the research, estimated to be four years. With respect to any inventions arising from the research, OPBG agreed to grant us an exclusive license to any such inventions, the terms of which will be set forth in a separate agreement. In addition, OPBG granted us paid-up, worldwide co-exclusive licenses for non-commercial development of OPBG's CD19 and CAR.GD2 CAR T technologies, as well as paid-up, worldwide exclusive licenses to commercialize its CD19 and CAR.GD2 CAR T technologies, each to be governed by a separate agreement.

## Financial Operations Overview

### Grant Revenue

To date, we have only recognized revenue from government grants and we have not generated any product revenue. We have received funds from CPRIT, and the 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.

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 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. Our policy is to recognize revenue in accordance with ASC 605. See the discussion of "Collaboration Agreements" contained within Note 2 to the audited financial statements contained within Item 8 of this Annual Report.

### Cancer Research Institute of Texas (CPRIT)

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 were 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 resulted in a deferred liability or grant receivable. The CPRIT grant expired as of June 30, 2014. As discussed above, we have received notice of an additional \$16.9 million grant from CPRIT, the terms of which are in negotiation, to support additional studies of BPX-501.

### NIH Grant

During 2013, we entered into a grant agreement with the 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 \$1.4 million to date, of which \$1.2 million has been received. We accrue the revenue based on the costs incurred for the programs associated with the grant.

### 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, share-based compensation expense 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. See the discussion of “Research and Development” expenses contained within Note 2 to the audited financial statements contained within Item 8 of this Annual Report.

Table of Contents

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. 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 outcomes of our clinical trials;
- 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.

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 ongoing scientific and clinical success of each product candidate, as well as an assessment of each product candidate's commercial potential.

We expect our research and development expenses to increase over the next several years as we progress our business plan which includes conducting ongoing and new clinical trials for BPX-501, BPX-601 and BPX-701 and advancing additional product candidates into clinical development, manufacturing clinical trial and preclinical study materials, expanding our research and development and process development and optimization efforts, seeking regulatory approvals for our product candidates that successfully complete clinical trials, and hiring additional personnel to support our research and development efforts.

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

	2016	2015	2014	2013	Total Inception Through December 31, 2016
Program	(in thousands)				
BPX-501	\$26,140	\$13,602	\$6,041	\$3,062	\$51,771
BPX-601	3,602	940	—	—	4,542
BPX-701	1,899	1,093	—	—	2,992
General	19,622	17,926	6,030	4,837	61,251
Total	\$51,263	\$33,561	\$12,071	\$7,899	\$120,556

Table of Contents

## 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, 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, and the potential commercialization of our product candidates. Additionally, if and when we believe a regulatory approval for the first product candidate appears likely, we anticipate an increase in payroll and related expenses as a result of our preparation for commercial operations, especially as it relates to the sales and marketing of our product candidates.

## Income Taxes

We did not recognize any income tax expense for the years ended December 31, 2016, 2015 and 2014.

## Other Income (Expense)

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

## Results of Operations

## Comparison of the Years Ended December 31, 2016 and 2015

The following table sets forth our results of operations for the years ended December 31, 2016 and 2015:

	Year ended December 31,		
	2016	2015	Change
	(in thousands)		
Grant revenues	\$388	\$282	\$106
Operating expenses:			
Research and development	51,263	33,561	17,702
License fees	580	3,184	(2,604 )
General and administrative	16,925	12,672	4,253
Total operating expenses	68,768	49,417	19,351
Loss from operations	(68,380 )	(49,135 )	(19,245 )
Other income (expense):			
Interest income	909	641	268
Interest expense	(1,760 )	(12 )	(1,748 )
Loss on disposition of fixed assets	(10 )	(42 )	32
Total other income (expense)	(861 )	587	(1,448 )
Net loss	\$(69,241 )	\$(48,548 )	\$(20,693 )

## Grant Revenues

Grant revenues were comparable in the years ended December 31, 2016 and 2015, and were comprised of our grant from the NIH.

## Research and Development Expenses

Research and development expenses were \$51.3 million and \$33.6 million for the years ended December 31, 2016 and 2015, respectively. During 2016, enrollment in our clinical trials for BPX-501 increased, compared with the previous year, resulting in additional clinical trials and manufacturing expenses. In addition, we conducted process development and optimization work on BPX-501 in preparation of manufacturing start-up activities in our U.S. facility.

The \$17.7 million increase in research and development expenses for the twelve months ended December 31, 2016, was primarily due to an increase in costs related to BPX-501 of approximately \$12.5 million. The increased costs related to BPX-501 include increases of, approximately \$1.3 million in clinical development activities, due to increased patient enrollment in our clinical trials, increases of





Table of Contents

approximately \$7.5 million in manufacturing costs as a result of increased patient enrollment in our clinical trials and process development and optimization costs related to the startup of manufacturing in our internal manufacturing facility, approximately \$2.3 million in regulatory and product characterization related studies of rimiducid and approximately \$1.4 million in other costs, primarily salaries and wages, related to the BPX-501 program. The increase in research and development expenses also included an increase of \$3.5 million in regulatory and other costs related to our preclinical product candidates, BPX-701 and BPX-601, primarily related to IND enabling activities; and an increase of approximately \$1.7 million in general research and development costs.

**License fees**

License fees were \$0.6 million and \$3.2 million for the years ended December 31, 2016 and 2015, respectively. The 2015 license fees included the license agreement with Agensys, as consideration for the rights granted to us under the agreement, whereby we paid Agensys a non-refundable upfront fee of \$3.0 million. For more information, see Notes 11 and 12 to the financial statements included herein for additional information about ARIAD and our license fees. If we are successful in our development activities under our existing and future licenses, we expect that our license fee expenses will increase in future years.

**General and Administrative Expenses**

General and administrative expenses were \$16.9 million and \$12.7 million for the years ended December 31, 2016 and 2015, respectively. The increase of \$4.2 million in 2016 was due to our overall growth and public company related costs. Share-based compensation expense increased approximately \$1.8 million, and other personnel-related expenses increased approximately \$1.0 million due to increases in personnel. Other costs, including legal and accounting expenses and costs related to facilities, insurance and travel increased approximately \$1.4 million.

**Other Income (Expense)**

Other income (expense) was \$(0.9) million and \$0.6 million for the years ended December 31, 2016 and 2015, respectively. The \$1.5 million of additional expense in 2016 was primarily due to \$1.8 million of interest expense related to the debt financing.

**Comparison of the Years Ended December 31, 2015 and 2014**

The following table sets forth our results of operations for the years ended December 31, 2015 and 2014:

	Year ended December 31,		
	2015	2014	Change
	(in thousands)		
Grant revenues	\$282	\$1,780	\$(1,498 )
Operating expenses:			
Research and development	33,561	12,071	21,490
License fees	3,184	—	3,184
ARIAD license restructuring	—	43,212	(43,212 )
General and administrative	12,672	4,335	8,337
Total operating expenses	49,417	59,618	(10,201 )
Loss from operations	(49,135 )	(57,838 )	8,703
Other income (expense):			
Interest income	641	35	606
Interest expense	(12 )	(1,791 )	1,779
Change in fair value of warrant liability	—	(24,371 )	24,371
Loss on disposition of fixed assets	(42 )	—	(42 )
Total other income (expense)	587	(26,127 )	26,714
Net loss	\$(48,548 )	\$(83,965 )	\$35,417

**Grant Revenues**

Grant revenues were \$0.3 million and \$1.8 million for the years ended December 31, 2015 and 2014, respectively. The decrease in grant revenues in 2014 was primarily due to the expiration of our grant award from CPRIT in June 2014.



## Table of Contents

### Research and Development Expenses

Research and development expenses were \$33.6 million and \$12.1 million for the years ended December 31, 2015 and 2014, respectively. The \$21.5 million increase in research and development expenses for the twelve months ended December 31, 2015, was due to an increase in costs related to BPX-501 of \$7.6 million, primarily due to the increase in clinical and manufacturing costs as a result of increased patient enrollment in our clinical trials. The increase in research and development expenses was also due to an increase of \$2.0 million in costs related to our product candidates, BPX-701 and BPX-601, primarily related to IND enabling activities; and an increase of \$11.9 million in general research and development costs comprised of \$6.3 million in personnel costs, \$2.7 million in allocated overhead costs and \$2.9 million in other costs.

### Reclassifications

Certain research and development indirect costs, including facilities and overhead, were previously included in general and administrative costs. These research and development indirect costs are included in research and development expense in the year ended December 31, 2015. The amounts for the year ended December 31, 2014 have been reclassified to conform to the current year presentation. The effect of the reclassification of the results for the twelve months ended December 31, 2014 was to increase research and development expense and reduce general and administrative expense by \$1.1 million with no change in total operating expense or net loss.

### License fees

License fees were \$3.2 million for the year ended December 31, 2015, compared to no license fees in 2014. The increase in license fees was primarily due to our new license agreement with Agensys, as consideration for the rights granted to us under the agreement, whereby we paid Agensys a non-refundable upfront fee of \$3.0 million. For more information, see Note 12 to the financial statements included herein.

### ARIAD License Restructuring

On October 3, 2014, we entered into an omnibus amendment agreement with ARIAD, under which we 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 our common stock that ARIAD held. In connection with the amendment, we made an initial payment of \$15.0 million and issued a promissory note to ARIAD for a principal amount of \$35.0 million. In December 2014 following our IPO, we paid the remaining \$35.0 million and ARIAD returned all 677,463 shares of our common stock 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 our common stock for \$5.1 million, and interest expense of \$1.7 million. We have recorded the returned shares of common stock as treasury stock. For more information, see Note 11 to the financial statements included herein.

### General and Administrative Expenses

General and administrative expenses were \$12.7 million and \$4.3 million for the years ended December 31, 2015 and 2014, respectively. The increase of \$8.4 million in 2015 was due to our overall growth and public company related costs, including an increase in personnel, legal and accounting expenses and costs related to facilities, insurance and travel.

### Other Income (Expense)

Other income (expense) was \$0.6 million and \$(26.1) million for the years ended December 31, 2015 and 2014, respectively. The \$26.7 million decrease in other expense in 2015 was primarily due to the change in fair value of a warrant liability of \$24.4 million and imputed interest expense from the ARIAD license restructuring of \$1.7 million. In connection with our August 2014 issuance of Series C convertible preferred stock, we 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 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. As all the warrants were either exercised or expired in December 2014, there were no future charges in connection with the warrants in 2015. Interest income in 2015 was a result of substantially higher levels of cash and investments.

## 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 grants. We have not generated any revenue from the sale of any products. As of December 31, 2016, we had cash, cash equivalents, restricted cash and investment securities of \$113.4 million. Cash in excess of

## Table of Contents

immediate requirements is invested in accordance with our investment policy, primarily with a view to liquidity and capital preservation.

On January 15, 2016, we filed a shelf Registration Statement on Form S-3 (File No. 333-209012), or the Shelf Registration Statement, to enable us to sell securities from time to time as described in the prospectus in one or more offerings up to a total aggregate offering price of \$150,000,000. The SEC declared the Shelf Registration Statement effective on February 1, 2016.

On March 10, 2016, we entered into a term loan arrangement with Hercules, as agent and lender and borrowed \$15.0 million on the closing date. We borrowed an additional \$5.0 million on September 15, 2016 and the remaining \$10.0 million on March 8, 2017. We intend to use the proceeds to complete the build-out of our manufacturing facilities, and for general corporate purposes. We are required to make monthly interest only payments through March 2018.

Thereafter, we are required to repay the loan over the remaining term, through its final maturity date of March 1, 2020. We incurred issuance costs of \$0.2 million and facility charges of \$2.1 million, which are payable at the earlier of the repayment of the loan in full or the final maturity date. The \$2.3 million debt issuance costs are being recognized over the term of the loan as additional interest expense. We will pay interest on the loan at the greater of either (i) 9.35% plus the prime rate as reported in the Wall Street Journal minus 3.5% and (ii) 9.35%. For additional information about the loan, see Note 7 to the audited financial statements included herein.

### 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. In addition, we expect to use capital to expand our manufacturing capabilities.

The successful development of any of our product candidates 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, but not limited to, the uncertainty of:

- successful enrollment in, and successful completion of, clinical trials;
- receipt of marketing approvals from applicable regulatory authorities;
- making arrangements with third-party manufacturers;
- obtaining and maintaining patent and trade secret protection and regulatory exclusivity;
- launching commercial sales of our products, if and when approved, whether alone or in collaboration with others; and
- market acceptance of our products, if and when approved;

• successfully negotiating reimbursement for our products from various third-party payors.

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, that 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.

Table of Contents

## 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, 2016 will enable us to fund our operating expenses and capital expenditure requirements through at least the first quarter of 2018. 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, BPX-701 and BPX-601 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 if they successfully complete clinical trials;
- establish sales, marketing and distribution infrastructure and scale-up manufacturing capabilities to commercialize any product candidates that may receive regulatory approval;
- build out European operations to support our product development and commercialization plans for BPX-501 and potentially other product candidates;
- 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 being a public company.

## Cash Flows

The following table sets forth a summary of our cash flows for the years ended December 31, 2016, 2015 and 2014:

	Year ended December 31,		
	2016	2015	2014
	(in thousands)		
Net cash used in operating activities	\$(50,441)	\$(35,726 )	\$(57,308 )
Net cash provided by (used in) investing activities	2,052	(86,453 )	(804 )
Net cash provided by financing activities	20,928	818	238,546
Net cash inflow (outflow)	\$(27,461)	\$(121,361)	\$180,434

## Operating Activities

Net cash used in operating activities of \$50.4 million for the year ended December 31, 2016, was comprised of a net loss of \$69.2 million, which included non-cash depreciation expense of \$2.3 million, amortization of deferred financing costs of \$0.4 million, amortization of premium on investment securities of \$0.5 million and share-based compensation expense of \$12.3 million. Reported net loss also includes approximately \$1.0 million excess of reported rent expense over cash rent paid to our landlord, included on our Balance Sheet as deferred rent. Net cash used in operating activities also included the effect of changes in asset and liability accounts, including a decrease in interest and other receivables of \$0.1 million, a decrease in prepaid and other current assets of \$0.9 million, an increase in accounts payable of \$0.9 million and an increase in accrued liabilities of \$1.3 million.

Net cash used in operating activities of \$35.7 million for the year ended December 31, 2015, was comprised of a net loss of \$48.5 million, which included depreciation expense of \$1.2 million and share-based compensation expense of \$8.4 million. Net cash used in operating activities was also comprised of the following primary components: a decrease in interest and other receivables of \$0.1 million, an increase in prepaid expenses and other current assets of \$1.1 million, a decrease in other assets of \$0.2 million, an increase in accounts payable of \$0.9 million, an increase in accrued liabilities \$2.8 million, and an increase in deferred costs of \$0.4 million.



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

Table of Contents

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.

**Investing Activities**

Net cash used in investing activities for the year ended December 31, 2016 was \$2.1 million, which was derived from proceeds from the sale of investment securities of \$42.5 million, offset by the purchases of investment securities of \$33.3 million, and purchases of property and equipment of \$7.2 million.

Net cash used in investing activities for the year ended December 31, 2015 was \$86.5 million, which was derived from the purchases of property and equipment of \$5.4 million and the purchase of investment securities of \$101.6 million, offset by proceeds from the sale of securities of \$20.6 million.

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.

**Financing Activities**

Net cash provided by financing activities for the year ended December 31, 2016 was \$20.9 million, which was primarily derived from proceeds of \$20.0 million received from borrowings on long-term debt, \$0.8 million from the proceeds of the exercise of stock options and \$0.3 million from the proceeds from employee purchases of common stock under the ESPP offset by \$0.2 million in debt issuance costs.

Net cash provided by financing activities for the year ended December 31, 2015 was \$0.8 million, which was derived from proceeds of \$0.5 million from the exercise of stock options and \$0.3 million proceeds from employee purchases of common stock under the ESPP.

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 December 2014 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 8 to the audited Financial Statements included herein.

**Contractual Obligations**

Our contractual obligations as of December 31, 2016 were as follows:

(in thousands)

	Total Commitments	Less Than 1 Year	1 - 3 Years	3 - 5 Years	More Than 5 Years
License agreements (1)	\$65,652	\$1,158	\$7,703	\$15,656	\$ 41,135
Long-term debt obligations (2)	21,390	1,787	15,953	3,650	—
Operating lease agreements (3)	13,753	1,982	4,120	2,202	5,449
Manufacturing build-out obligation (4)	10,079	10,079	—	—	—
Research collaborations (5)	4,375	1,094	2,187	1,094	—
Manufacturing arrangements (6)	2,993	2,214	779	—	—
Sponsored research agreements (7)	2,342	1,000	1,342	—	—
Equipment capital lease agreements (8)	273	59	118	96	—
Total contractual obligations	\$ 120,857	\$ 19,373	\$ 32,202	\$ 22,698	\$ 46,584

License agreements - 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 (1) clinical and regulatory milestones regarding the products covered by the licenses. The obligations listed in the table above represent estimates of when the milestones will be achieved. The milestones may not be completed when estimated or at all. See Note 12 to the financial statements included herein.

(2) Long-term debt obligations - Obligations under our debt facility. See Note 7 to the financial statements included herein.

(3) Operating lease agreements - The amounts above are comprised of one five-year lease agreement and one 11-year lease agreement. The first lease expires on January 31, 2020 and the second lease expires on August 31, 2026. See Note 12 to the financial statements included herein.

(4) Manufacturing build-out obligation - We entered into a construction contract to build-out our manufacturing facilities. The obligation listed in the table above represents the remaining agreed upon costs.

## Table of Contents

- (5) Research collaborations - We entered into a research collaboration with OPBG with commitments over 4 years. See Note 12 to the financial statements included herein.
- (6) Manufacturing arrangements - We have entered into a number of manufacturing service arrangements with various terms. The obligations listed in the table above represent estimates of when certain services will be performed.
- (7) Sponsored research agreements - We have entered into two sponsored research agreements to undertake research which is of mutual interest to all parties. The commitments range from one to three years.
- Equipment capital lease agreements - We have entered into a number of office equipment lease agreements with (8) various terms. The commitments include equipment, maintenance and supplies. See Note 12 to the financial statements included herein.

We have entered and will enter into other contracts in the normal course of business with third-party manufacturers, contract research organizations for clinical trials and other vendors for other services and products for operating purposes. These agreements generally provide for termination or cancellation, and, other than for costs already incurred, are not included in the table above.

### 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

To date, we have only recognized revenue from government grants and we have not generated any product revenue. We have received funds from the CPRIT, and the 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.

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. See discussion of "Collaboration Agreements" in Note 2 to the audited financial statements included in this Annual Report.

### 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 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.

#### Contract Manufacturing Services

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

Table of Contents

## Share-Based Compensation

We account for share-based compensation by calculating the fair value of equity awards on the date of grant. We calculate the fair value of stock options using the Black-Scholes pricing model, which requires a number of estimates, including the expected lives of awards, interest rates, stock volatility and other assumptions. Restricted stock is measured based on the fair market value of the underlying stock on the date of grant. If the awards are classified as liability awards, the fair value is remeasured at each reporting date and the compensation expense is adjusted accordingly. Additionally, we apply a forfeiture rate to estimate the number of grants that will ultimately vest, as applicable, and adjust the expense as these awards vest. All of our current equity awards are service based awards and the share-based compensation cost is being recognized over the requisite service period of the awards on a straight-line basis. Our share-based compensation expense has increased due to the growth in the number of our employees and also due to the increase in the valuation of equity awards as a result of becoming a public company in December of 2014.

The following table sets forth the share-based compensation expense included in our results of operations for the years ended December 31, 2016, 2015 and 2014:

	Year Ended December 31,		
	2016	2015	2014
	(in thousands)		
General and administrative	\$6,681	\$4,832	\$386
Research and development	5,656	3,577	525
Total	\$12,337	\$8,409	\$911

## 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, 2016, 2015 and 2014, we had no uncertain tax positions and no interest or penalties have been charged to us for the years ended December 31, 2016, 2015 and 2014. 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 2005 through 2016 remain open to examination by the U.S. Internal Revenue Service.

## Recently Issued Accounting Pronouncements

See Note 2 to the Notes to Consolidated Financial Statements in "Item 8 - Financial Statements and Supplementary Data" in this Annual Report for discussion regarding recent accounting pronouncements.

## 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

Table of Contents

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.

Subject to certain conditions set forth in the JOBS Act, as an “emerging growth company,” we intend to rely on certain of these exemptions including without limitation with respect to, (1) providing an auditor’s attestation report on our system of internal controls over financial reporting pursuant to Section 404(b) of the Sarbanes-Oxley Act and (2) complying with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor’s report providing additional information about the audit and the financial statements, known as the auditor discussion and analysis. We will remain an “emerging growth company” until the earliest of (a) the last day of the fiscal year in which we have total annual gross revenues of \$1 billion or more, (b) the last day of our fiscal year following the fifth anniversary of the date of the completion of our initial public offering, (c) the date on which we have issued more than \$1 billion in non-convertible debt during the previous three years or (d) the date on which we are deemed to be a large accelerated filer under the rules of the SEC.

**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 realize income from our investments without assuming significant risk. To achieve our objectives, we invest our cash allocated to fund our short-term liquidity requirements with prominent financial institutions in bank depository accounts and institutional money market funds. We invest the remainder of our cash in corporate debt securities and municipal bonds rated at least A quality or equivalent, U.S. Treasury notes and bonds and U.S. and state government agency-backed securities. As of December 31, 2016, we had cash, cash equivalents, restricted cash and investment in marketable securities of \$113.4 million.

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, 2016:

	Page
<u>Report of Independent Registered Public Accounting Firm</u>	<u>72</u>
<u>Balance Sheets</u>	<u>73</u>
<u>Statements of Operations and Comprehensive Loss</u>	<u>74</u>
<u>Statements of Redeemable and Convertible Preferred Stock and Stockholders’ Equity (Deficit)</u>	<u>75</u>
<u>Statements of Cash Flows</u>	<u>76</u>
<u>Notes to the Financial Statements</u>	<u>77</u>



Table of Contents

Report of Independent Registered Public Accounting Firm

The Board of Directors and Shareholders of Bellicum Pharmaceuticals, Inc.

We have audited the accompanying balance sheets of Bellicum Pharmaceuticals, Inc. as of December 31, 2016 and 2015, and the related statements of operations and comprehensive loss, redeemable and convertible preferred stock and stockholders' equity (deficit) and cash flows for each of the three years in the period ended December 31, 2016. 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, 2016 and 2015, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2016, in conformity with U.S. generally accepted accounting principles.

/s/ Ernst & Young LLP

Houston, Texas

March 13, 2017

Table of Contents

Bellicum Pharmaceuticals, Inc.

Balance Sheets

(in thousands, except for par value and share data)

	December 31, 2016	December 31, 2015
<b>ASSETS</b>		
Current assets:		
Cash and cash equivalents	\$33,140	\$70,241
Investment securities, available for sale	70,632	23,820
Accounts receivable, interest and other receivables	334	440
Prepaid expenses and other current assets	1,504	2,389
Total current assets	105,610	96,890
Investment securities, available for sale - long-term	—	56,304
Property and equipment, net	16,504	6,882
Restricted cash	9,640	—
Other assets	283	330
<b>TOTAL ASSETS</b>	<b>\$132,037</b>	<b>\$160,406</b>
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Current liabilities:		
Accounts payable	\$3,623	\$2,106
Accrued expenses and other current liabilities	9,363	5,080
Current maturity of long-term debt	1,787	—
Current portion of capital lease obligations	21	13
Current portion of deferred rent	319	246
Total current liabilities	15,113	7,445
Long-term liabilities:		
Long-term debt	18,436	—
Capital lease obligations	141	118
Deferred rent	1,773	826
<b>TOTAL LIABILITIES</b>	<b>35,463</b>	<b>8,389</b>
Commitments and contingencies: (Note 12)		
Stockholders' Equity:		
Preferred stock: \$0.01 par value; 10,000,000 shares authorized: no shares issued and outstanding	—	—
Common stock: \$0.01 par value; 200,000,000 shares authorized at December 31, 2016 and 2015; 27,833,028 shares issued and 27,155,565 shares outstanding at December 31, 2016; 27,609,344 shares issued and 26,931,881 shares outstanding at December 31, 2015	278	276
Treasury stock: 677,463 shares held at December 31, 2016 and 2015	(5,056 )	(5,056 )
Additional paid-in capital	332,068	318,591
Accumulated other comprehensive income (loss)	17	(302 )
Accumulated deficit	(230,733 )	(161,492 )
Total stockholders' equity	96,574	152,017
<b>TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY</b>	<b>\$132,037</b>	<b>\$160,406</b>
The accompanying notes are an integral part of these financial statements.		



Table of Contents

Bellicum Pharmaceuticals, Inc.

Statements of Operations and Comprehensive Loss

(in thousands, except share and per share amounts)

	Year Ended December 31,		
	2016	2015	2014
REVENUES			
Grants	\$388	\$282	\$1,780
Total revenues	388	282	1,780
OPERATING EXPENSES			
Research and development	51,263	33,561	12,071
License fees	580	3,184	—
ARIAD restructuring costs	—	—	43,212
General and administrative	16,925	12,672	4,335
Total operating expenses	68,768	49,417	59,618
LOSS FROM OPERATIONS	(68,380 )	(49,135 )	(57,838 )
OTHER INCOME (EXPENSE)			
Interest income	909	641	35
Interest expense	(1,760 )	(12 )	(1,791 )
Loss on disposal of assets	(10 )	(42 )	—
Change in fair value of warrant liability	—	—	(24,371 )
Total other income (expense)	(861 )	587	(26,127 )
NET LOSS	\$(69,241 )	\$(48,548 )	\$(83,965 )
Preferred stock dividends	—	—	(1,432 )
Net loss attributable to common stockholders	\$(69,241 )	\$(48,548 )	\$(85,397 )
Net loss per common share attributable to common shareholders, basic and diluted	\$(2.57 )	\$(1.84 )	\$(34.04 )
Weighted-average shares outstanding-basic and diluted	26,950,906	26,346,603	2,508,960
Net Loss	\$(69,241 )	\$(48,548 )	\$(83,965 )
Other comprehensive loss:			
Unrealized gain (loss) on securities, net	319	(302 )	—
Comprehensive loss	\$(68,922 )	\$(48,850 )	\$(83,965 )
The accompanying notes are an integral part of these financial statements.			

Table of Contents

Bellicum Pharmaceuticals, Inc.

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

Years Ended December 31, 2016, 2015 and 2014

(amounts in thousands, except share data)

	Series A		Series B		Series C	Common Stock		Treasury Stock		Addition	
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Paid-In Capital
Balance, January 1, 2014	2,544,539	\$7,634	6,563,283	\$32,292	—	\$—	1,725,992	\$17	—	\$—	\$810
Share-based compensation											911
Issuance of restricted stock grant						117,647	1				(1
Exercise of stock options						12,615					11
Issuance of common stock in an IPO, net of issuance costs						8,452,500	85				146,218
Issue Series B preferred stock, net of issuance costs			1,582,706	7,320							
Issue Series C preferred stock, net of issuance costs					10,091,743	42,074					
Exercise of Series C warrants, net of issuance costs					6,524,195	72,187					
Exercise of common warrants						510,524	5				245
Accretion of Series B dividend				1,432							(1,432
Payment of Series B dividend				(173	)						
Repurchase of common stock held by ARIAD								(677,463)	(5,056	)	

Edgar Filing: BELLICUM PHARMACEUTICALS, INC - Form 10-K

Conversion of preferred stock	(2,544,539)	(7,634 )	(8,145,989)	(40,871 )	(16,615,938)	(14,010,777)	163			162,603
Net loss										
Balance, December 31, 2014	—	\$—	—	\$—	—	\$—27,050,055	\$271	(677,463)	\$(5,056)	\$309,36
Share-based compensation										8,409
Exercise of stock options						182,238	1			481
Issuance of common stock - Employee Stock Purchase Plan						21,690				347
Exercise of common warrants						355,361	4			(4
Other Comprehensive loss										(7
Balance, December 31, 2015	—	\$—	—	\$—	—	\$—27,609,344	\$276	(677,463)	\$(5,056)	\$318,59
Share-based compensation										12,337
Exercise of stock options						190,055	2			771
Issuance of common stock - Employee Stock Purchase Plan						33,629				369
Comprehensive income (loss)										
Balance, December 31, 2016	—	\$—	—	\$—	—	\$—27,833,028	\$278	(677,463)	\$(5,056)	\$332,06

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

Table of Contents

Bellicum Pharmaceuticals Inc.  
 Statements of Cash Flows  
 (in thousands)

	Year Ended December 31,		
	2016	2015	2014
<b>CASH FLOWS FROM OPERATING ACTIVITIES:</b>			
Net loss	\$(69,241)	\$(48,548)	\$(83,965)
Adjustments to reconcile net loss to net cash used in operating activities:			
Share-based compensation	12,337	8,409	911
Depreciation expense	2,306	1,199	667
Amortization of premium on investment securities, net	539	573	—
Amortization of lease liability	(119)	(94)	(89)
Amortization of deferred financing costs	422	—	—
Loss on disposal of property and equipment	10	42	—
Loss on disposition of investment securities	—	33	—
Change in fair value of warrant liability	—	—	24,371
Changes in operating assets and liabilities:			
Accounts receivable	106	(142)	448
Prepaid expenses and other current assets	885	(1,067)	(1,068)
Other assets	47	(185)	339
Accounts payable	931	897	659
Accrued liabilities and other	1,336	2,778	225
Deferred revenue – grants	—	(13)	13
Deferred rent	—	859	5
Deferred manufacturing costs	—	(467)	176
<b>NET CASH USED IN OPERATING ACTIVITIES</b>	<b>(50,441)</b>	<b>(35,726)</b>	<b>(57,308)</b>
<b>CASH FLOWS FROM INVESTING ACTIVITIES:</b>			
Proceeds from sale of investment securities	42,548	20,617	—
Purchases of investment securities	(33,276)	(101,649)	—
Purchases of property and equipment	(7,220)	(5,421)	(804)
<b>CASH PROVIDED BY (USED IN) INVESTING ACTIVITIES</b>	<b>2,052</b>	<b>(86,453)</b>	<b>(804)</b>
<b>CASH FLOWS FROM FINANCING ACTIVITIES:</b>			
Proceeds from debt	20,000	—	386
Payments on debt	—	—	(1,187)
Payment of debt issuance costs	(199)	—	—
Payment on capital lease obligations	(15)	(4)	—
Proceeds from issuance of common stock	—	—	160,609
Proceeds from exercise of stock options	773	482	—
Proceeds from issuance of common stock - ESPP	369	347	—
Payment of issuance costs on common stock	—	(7)	(14,242)
Proceeds from issuance of preferred stock	—	—	62,320
Payment of issuance costs on preferred stock	—	—	(3,524)
Proceeds from exercise of preferred warrants	—	—	39,145
Proceeds from exercise of common warrants	—	—	250
Payment for repurchase of common stock	—	—	(5,056)
Payment of preferred dividends	—	—	(155)
<b>NET CASH PROVIDED BY FINANCING ACTIVITIES</b>	<b>20,928</b>	<b>818</b>	<b>238,546</b>
<b>NET CHANGE IN CASH, CASH EQUIVALENTS AND RESTRICTED CASH</b>	<b>(27,461)</b>	<b>(121,361)</b>	<b>180,434</b>
<b>CASH, CASH EQUIVALENTS AND RESTRICTED CASH AT BEGINNING OF YEAR</b>	<b>70,241</b>	<b>191,602</b>	<b>11,168</b>

CASH, CASH EQUIVALENTS AND RESTRICTED CASH AT END OF YEAR	\$42,780	\$70,241	\$191,602
SUPPLEMENTAL CASH FLOW INFORMATION:			
Cash paid during the period for interest	\$1,136	\$—	\$1,767
NON-CASH INVESTING AND FINANCING ACTIVITIES			
Purchases of property and equipment in accounts payables and accrued liabilities	\$3,533	\$139	\$—
Leasehold improvements paid by landlord	\$1,139	\$—	\$—
Accrued debt issuance costs	\$1,390	\$—	\$—
Capital lease obligations incurred for equipment	\$46	\$135	\$—
Preferred stock dividends paid in common stock	\$—	\$—	\$3,196
Dividends accreted on preferred stock	\$—	\$—	\$1,432

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



Table of Contents

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 and TCR cell therapy. The Company has not generated any revenue from product sales to date and if the Company does not successfully commercialize any of the Company's product candidates, the Company will not be able to generate product revenue or achieve profitability. As of December 31, 2016, the Company had an accumulated deficit of \$230.7 million.

The Company is subject to risks common to companies in the biotechnology industry and 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, manage the growth of the organization, obtain additional financing necessary in order to develop, launch and commercialize its product candidates, and compete 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).

**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.

**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, 2017, and a \$1.3 million research grant from the National Institutes of Health (NIH) covering the period from April 2013 to March 2017. 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 10).

**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.

**Investment Securities**

Consistent with its investment policy, the Company invests its cash allocated to fund its short-term liquidity requirements with prominent financial institutions in bank depository accounts and institutional money market funds. The Company invests the remainder of its cash in corporate debt securities and municipal bonds rated at least A quality or equivalent, U.S. Treasury notes and bonds and U.S. and state government agency-backed securities.

The Company determines the appropriate classification of investment securities based on whether they represent the investment of funds available for current operations, as defined in ASC 210-10-45-1 and ASC 210-10-45-2. The Company reevaluates its classification as of each balance sheet date. All investment securities owned are classified as available-for-sale. The cost of securities

Table of Contents

Notes to the Financial Statements

sold is based on the specific identification method. Investment securities are recorded as of each balance sheet date at fair value, with unrealized gains and, to the extent deemed temporary, unrealized losses reported as accumulated other comprehensive gain (loss), a separate component of stockholders' equity. Interest and dividend income on investment securities, accretion of discounts and amortization of premiums and realized gains and losses are included in interest income in the statements of operations and comprehensive income (loss).

An investment security is considered to be impaired when a decline in fair value below its cost basis is determined to be other than temporary. The Company evaluates whether a decline in fair value of an investment security is below its cost basis is other than temporary using available evidence. In the event that the cost basis of the investment security exceeds its fair value, the Company evaluates, among other factors, the amount and duration of the period that the fair value is less than the cost basis, the financial health of and business outlook for the issuer, including industry and sector performance, and operational and financing cash flow factors, overall market conditions and trends, the Company's intent to sell the investment security and whether it is more likely than not the Company would be required to sell the investment security before its anticipated recovery. If a decline in fair value is determined to be other than temporary, the Company records an impairment charge in the statement of operations and comprehensive loss and establishes a new cost basis in the investment.

**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. Leasehold improvements are amortized over the shorter of the estimated useful life or the remaining lease term.

**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, 2016, 2015 and 2014.

**Debt Issuance Costs**

Costs related to debt issuance are presented in the balance sheet as a direct deduction from the carrying amount of the debt liability, consistent with debt discounts and are amortized using the effective interest method. Amortization of debt issuance costs are included in interest expense.

**Deferred Rent and 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.

**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, as described further in Note 4. The Company believes the recorded values of its financial instruments, including cash and cash equivalents, accounts payable and accrued liabilities approximate their fair values due to the short-term nature of these instruments.

**Financial Instruments and Credit Risks**

Financial instruments that potentially subject the Company to credit risk include cash and cash equivalents, investment securities, 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.

Table of Contents

Notes to the Financial Statements

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.

Clinical Trials

The Company estimates its clinical trial expense accrual for a given period based on the number of patients enrolled at each site, estimated cost per patient, and the length of time each patient has been in the trial, less amounts previously billed. These accruals are recorded in accrued expenses and other current liabilities, and the related expense is recorded in research and development expense.

Research and Development

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. The Company accrues for costs incurred as the services are being provided by monitoring the status of the clinical trial or project and the invoices received from its external service providers. The Company estimates depend on the timeliness and accuracy of the data provided by the vendors regarding the status of each project and total project spending. The Company adjusts its 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.

Collaboration Agreements

The Company enters into collaboration agreements that include varying arrangements regarding which parties perform and bear the costs of research and development activities. The Company may share the costs of research and development activities with a collaborator, or the Company may be reimbursed for all or a significant portion of the costs of the Company's research and development activities. The Company records its internal and third-party development costs associated with these collaborations as research and development expenses. When the Company is entitled to reimbursement of all or a portion of the research and development expenses that it incurs under a collaboration, the Company records those reimbursable amounts as a deduction to the research and development expenses. If the collaboration is a cost-sharing arrangement in which both the Company and its collaborator perform development work and share costs, the Company also recognizes, as research and development expenses in the period when its collaborator incurs development expenses, the portion of the collaborator's development expenses that the Company is obligated to reimburse.

Reclassifications

Certain research and development indirect costs, including facilities and overhead, were previously included in general and administrative costs. These research and development indirect costs are included in research and development expense for the year ended December 31, 2016 and 2015. The results for the year ended December 31, 2014 have been reclassified to conform to the current year presentation. The effect of the reclassification of the results for the year ended December 31, 2014 was to increase research and development expense and reduce general and administrative expense by \$1.1 million with no change in total operating expense or net loss.

Contract Manufacturing Services

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

79

---

Table of Contents

Notes to the Financial Statements

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. 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 IPO on December 23, 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, 2016, 2015 and 2014, the Company had no uncertain tax positions and no interest or penalties have been charged to the Company for the years ended December 31, 2016, 2015 and 2014. 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 2016 remain open to examination by the Internal Revenue Service.

Comprehensive 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. Components of comprehensive income (loss) includes, among other items, unrealized gains and losses on the changes in fair value of investments. These components are added, net of their related tax effect, to the reported net income (loss) to arrive at comprehensive income (loss). The components of accumulated other comprehensive loss at December 31, 2016 and 2015, on the Company's balance sheet was comprised of the net unrealized holding losses on the Company's investment securities. See Note 4 for further detail of the unrealized holding gains and losses on the Company's investment securities. 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.



Table of Contents

## Notes to the Financial Statements

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.

	Number of shares		
	December 31, 2016	December 31, 2015	December 31, 2014
Options to purchase common stock	4,532,120	3,628,973	2,733,793
Unvested shares of restricted stock	58,825	88,236	117,647
Total common stock equivalents	4,590,945	3,717,209	2,851,440

**Application of New Accounting Standards**

ASU No. 2014-15, "Disclosures of Uncertainties about an Entity's Ability to Continue as a Going Concern," became effective for the Company in 2016. ASU No. 2014-15 requires management to evaluate the Company's ability to meet its obligations as they become due within one year after the date that financial statements are issued. Accordingly, management has assessed the Company's ability to continue as a going concern through March 31, 2018. In making its assessment, management evaluated the Company's liquid assets, the Company's obligations expected to become payable within the period, and the probability of other conditions and events, and concluded that the Company's ability to continue as a going concern is not in substantial doubt.

ASU No. 2016-18, "Statement of Cash Flows (Topic 230): Restricted Cash," requires restricted cash to be included with cash and cash equivalents when reconciling the beginning and ending amounts on the statement of cash flows, and requires additional disclosures in the notes to the financial statements. The Company adopted this standard during 2016. See Note 3 to the financial statements included herein.

ASU No. 2015-03, "Simplifying the Presentation of Debt Issuance Costs," requires that debt issuance costs related to a recognized debt liability be presented in the balance sheet as a direct deduction from the carrying amount of that debt liability, consistent with debt discounts. See Note 7 to the financial statements included herein.

ASU No. 2015-17, "Balance Sheet Classification of Deferred Taxes," requires that deferred income tax liabilities and assets be classified as noncurrent in a classified statement of financial position. The Company adopted this standard as of December 31, 2015, prospectively. See Note 13 to the financial statements included herein.

**New Accounting Requirements and Disclosures**

In January 2016, the FASB issued ASU No. 2016-01, "Recognition and Measurement of Financial Assets and Financial Liabilities." ASU 2016-01 requires that most equity investments be measured at fair value, with subsequent changes in fair value recognized in net income. The pronouncement also impacts financial liabilities under the fair value option and the presentation and disclosure requirements for financial instruments. This pronouncement is effective for fiscal years, and interim periods within those years, beginning after December 15, 2017, and early adoption is not permitted. The Company does not believe that the adoption of this pronouncement will have a material impact on the Company's financial statements.

In February 2016, the FASB issued ASU No. 2016-02, "Leases," which requires companies that lease assets to recognize a right-of-use asset and a lease liability, initially measured at the present value of the lease payments, in its balance sheet. The pronouncement will also require additional disclosures about the amount, timing and uncertainty of cash flows arising from leases. This pronouncement is effective for fiscal years, and interim periods within those years, beginning after December 15, 2018, and early adoption is permitted. The Company is currently evaluating the impact of this pronouncement on the Company's financial statements.

In March 2016, the FASB issued ASU No. 2016-09, "Compensation-Stock Compensation," which simplifies accounting for share-based compensation arrangements, primarily as it relates to accounting for the income tax effects of share-based compensation. Under the pronouncement, an entity can make an entity-wide accounting policy decision to either estimate the number of awards that are expected to vest (current GAAP) or account for forfeitures as they occur. The pronouncement is effective for annual periods beginning after December 31, 2016, with earlier adoption permitted. The Company does not believe the adoption of this standard will have a material impact on the Company's financial statements.

In August 2016, the FASB issued ASU 2016-15, "Classification of Certain Cash Receipts and Cash Payments," which provides guidance on the classification of certain cash receipts and payments in the statement of cash flows. The pronouncement is effective for

Table of Contents

## Notes to the Financial Statements

annual periods beginning after December 15, 2017, and interim periods within those annual periods. Earlier application is permitted in any interim or annual period. The Company does not believe the adoption of this standard will have a material impact on the Company's financial statements.

**NOTE 3 - CASH, CASH EQUIVALENTS AND RESTRICTED CASH**

As of December 31, 2016, the Company maintained \$9.6 million as restricted cash. The funds are being held with an escrow agent to cover the construction of certain manufacturing costs related to the facility lease. This amount is subject to the terms of the escrow agreement in the lease and the requirements specified therein. This amount may decrease as the Company and landlord authorize completion of certain aspects of the building improvements. See Note 12 to the financial statements included herein.

The following table provides a reconciliation of cash, cash equivalents and restricted cash reported within the balance sheets that sum to the total of the same such amounts shown in the statements of cash flows.

	December 31, 2016	December 31, 2015
	(in thousands)	
Cash and cash equivalents (1)	\$33,140	\$ 70,241
Restricted cash, noncurrent	9,640	—
Total cash, cash equivalents and restricted cash shown in the statements of cash flows	\$42,780	\$ 70,241

(1) As of December 31, 2016 and 2015, the Company invested approximately \$23.5 million and \$62.2 million, respectively, in cash equivalent instruments.

**NOTE 4 - FAIR VALUE OF MEASUREMENTS AND INVESTMENT SECURITIES**

The Company follows ASC, Topic 820, Fair Value Measurements and Disclosures, or ASC 820, for application to financial assets. ASC 820 defines fair value, provides a consistent framework for measuring fair value under GAAP and requires fair value financial statement disclosures. ASC 820 applies only to the measurement and disclosure of financial assets that are required or permitted to be measured and reported at fair value under other ASC topics (except for standards that relate to share-based payments such as ASC Topic 718, Compensation – Stock Compensation). The valuation techniques required by ASC 820 may be based on either observable or unobservable inputs. Observable inputs reflect readily obtainable data from independent sources, and unobservable inputs reflect the Company's market assumptions.

These inputs are classified into the following hierarchy:

Level 1 Inputs – quoted prices (unadjusted) in active markets for identical assets that the reporting entity has the ability to access at the measurement date;

Level 2 Inputs – inputs other than quoted prices included within Level 1 that are observable for the asset, either directly or indirectly; and

Level 3 Inputs – unobservable inputs for the assets.

The following tables present the Company's investment securities (including, if applicable, those classified on the Company's balance sheet as cash equivalents) that are measured at fair value on a recurring basis as of December 31, 2016 and 2015:

Table of Contents

## Notes to the Financial Statements

	Fair Value Measurements at Reporting Date Using			
	Balance at December 31, 2016 (in thousands)	Quoted prices in active markets for identical assets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
Cash Equivalents:				
Money market funds	23,459	23,459	—	—
U.S. government agency-backed securities	—	—	—	—
Total Cash Equivalents	\$23,459	\$23,459	\$ —	\$ —
Investment Securities:				
U.S. government agency-backed securities	\$25,908	\$ —	\$ 25,908	\$ —
Corporate debt securities	42,053	—	42,053	—
Municipal bonds	2,671	—	2,671	—
Total Investment Securities	\$70,632	\$ —	\$ 70,632	\$ —

	Fair Value Measurements at Reporting Date Using			
	Balance at December 2015 (in thousands)	Quoted prices in active markets for identical assets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
Cash Equivalents:				
Money market funds	\$52,714	\$52,714	\$ —	\$ —
U.S. government agency-backed securities	9,500	—	9,500	—
Total Cash Equivalents	\$62,214	\$52,714	\$ 9,500	\$ —
Investment Securities:				
U.S. government agency-backed securities	\$22,388	\$ —	\$ 22,388	\$ —
Corporate debt securities	51,547	—	51,547	—
Municipal bonds	6,189	—	6,189	—
Total Investment Securities	\$80,124	\$ —	\$ 80,124	\$ —

U.S. Treasury, U.S. government agency-backed securities, corporate debt securities and municipal bonds are valued based on various observable inputs such as benchmark yields, reported trades, broker/dealer quotes, benchmark securities and bids.

Management believes that the carrying value of the debt facility approximates its fair value, as the Company's debt facility bears interest at a rate that approximates prevailing market rates for instruments with similar characteristics. The fair value of the Company's debt facility is determined under Level 2 in the fair value hierarchy.

Table of Contents

## Notes to the Financial Statements

Investment securities, all classified as available-for-sale, consisted of the following as of December 31, 2016 and 2015:

Description	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Aggregate Estimated Fair Value
December 31, 2016	(in thousands)			
U.S. government agency-backed securities	\$25,906	\$ 7	\$ (5 )	\$ 25,908
Corporate debt securities	42,040	41	(28 )	42,053
Municipal bonds	2,669	2	—	2,671
Total	\$70,615	\$ 50	\$ (33 )	\$ 70,632
December 31, 2015				
U.S. government agency-backed securities	\$22,417	\$ 1	\$ (30 )	\$ 22,388
Corporate debt securities	51,807	1	(261 )	51,547
Municipal bonds	6,200	—	(11 )	6,189
Total	\$80,424	\$ 2	\$ (302 )	\$ 80,124

During the year ended December 31, 2016, the Company realized approximately \$6,700 of the unrealized loss at December 31, 2015. The Company's investment securities as of December 31, 2016, will reach maturity between January 2017 and January 2019, with a weighted-average maturity date in August 2017.

The Company has classified all of its available -for-sale investment securities, including those with maturities beyond one year, as current assets on the accompanying balance sheets based on the highly liquid nature of the investment securities and because these investment securities are considered available for use in current operations.

**NOTE 5 - PROPERTY AND EQUIPMENT**

Property and equipment consists of the following:

		December 31,	
		2016	2015
	Estimated Useful Lives	(in thousands)	
Leasehold improvements	5 years	\$12,131	\$4,092
Lab equipment	5 years	5,397	3,741
Office furniture	5 years	1,560	931
Manufacturing equipment	5 years	1,275	0
Computer and office equipment	3 to 5 years	623	401
Equipment held under capital leases	5 years	181	135
Software	3 years	85	109
Total		21,252	9,409
Less: accumulated depreciation		(4,748 )	(2,527 )
Property and equipment, net		\$16,504	\$6,882

During the years ended December 31, 2016, 2015, and 2014, the Company recorded \$2.3 million, \$1.2 million and \$0.7 million of depreciation expense, respectively. Leasehold improvements at December 31, 2016 includes \$2.5 million related to costs incurred by the landlord. Please refer to Note 12, "Commitments and contingencies," for further

information.

84

---

Table of Contents

## Notes to the Financial Statements

**NOTE 6 - ACCRUED EXPENSES AND OTHER CURRENT LIABILITIES**

Accrued expenses and other liabilities consist of the following:	December 31,	
	2016	2015
	(in thousands)	
Accrued construction costs	\$3,120	\$—
Accrued manufacturing costs	1,704	2,412
Accrued payroll	\$1,568	\$1,332
Accrued patient treatment costs	1,006	333
Accrued other	1,965	1,003
Total accrued expenses and other current liabilities	\$9,363	\$5,080

**NOTE 7 - DEBT**

On March 10, 2016 (the Closing Date), the Company entered into a Loan and Security Agreement (the Loan Agreement) with Hercules Capital, Inc., Hercules Technology II, L.P., and Hercules Technology III, L.P., or collectively, Hercules, as a lender, under which the Company borrowed \$15.0 million. The Company borrowed an additional \$5.0 million and \$10.0 million on September 15, 2016 and March 8, 2017, respectively. The total debt is secured by a lien covering substantially all of our assets, excluding intellectual property, but including proceeds from the sale, license, or disposition of our intellectual property. The Company intends to use the proceeds received under the Loan Agreement for funding the build-out of our manufacturing facilities and general corporate purposes. Please refer to Note 15, "Subsequent events" for further information.

The interest rate will be calculated at a rate equal to the greater of either (i) 9.35% plus the prime rate as reported in The Wall Street Journal minus 3.50%, or (ii) 9.35%. The interest rate on amounts borrowed under the Loan Agreement was 9.6% at December 31, 2016. Payments under the Loan Agreement are interest only for 18 months from the Closing Date, extendable to 24 months upon the Company achieving the Milestones. The interest only period will be followed by equal monthly payments of principal and interest amortized over a 30 months schedule through the maturity date of March 1, 2020 (the Loan Maturity Date); provided that if the Milestones are achieved, the Company will make equal monthly payments of principal and interest amortized over a 24 months schedule through the Loan Maturity Date. The remaining principal balance will be due and payable on the Loan Maturity Date. In addition, upon the Loan Maturity date or such earlier date specified in the Loan Agreement, a final payment equal to \$1,390,000 (the Final Facility Charge), plus, an additional facility charge of \$695,000, for an aggregate end-of-term charge of \$2,085,000. The Company's obligations under the Loan Agreement are secured by a security interest in substantially all of its assets, other than its intellectual property.

If the Company prepays the loan, including interest, prior to the date that is 24 months following March 10, 2016, it will pay Hercules a prepayment charge based on a prepayment fee equal to 2.00% of the amount prepaid; if the prepayment occurs thereafter, it will pay Hercules a prepayment charge based on a prepayment fee equal to 1.00% of the amount prepaid. The prepayment charge is also applicable upon the occurrence of a change of control of the Company. In addition to a prepayment charge, if any, the Company will pay Hercules the Final Facility Charge.

The Loan Agreement includes customary affirmative and restrictive covenants, but does not include any financial maintenance covenants, and also includes standard events of default, including payment defaults. Upon the occurrence of an event of default, a default interest rate of an additional 5% may be applied to the outstanding loan balance and Hercules may declare all outstanding obligations immediately due and payable and take such other actions as set forth in the Loan Agreement. The Company paid expenses related to the Loan Agreement of \$199,000, which, along with the Final Facility Charge of \$1,390,000, have been recorded as deferred financing costs, which offset long-term debt on the Company's balance sheet. Deferred financing costs of \$1,589,000 are being amortized over the term of the loan, and are included in interest expenses. During the year ended December 31, 2016, interest expense included \$422,000



of amortized deferred financing costs.

Table of Contents

## Notes to the Financial Statements

The total gross payments due under our debt arrangements are as follows:

Year	As of December 31, 2016 (in thousands)
2017	\$ 1,787
2018	7,590
2019	8,363
2020	3,650
Total	\$ 21,390

**NOTE 8 - COMMON STOCK, PREFERRED STOCK AND WARRANTS****Common Stock**

During the year ended December 31, 2014, the Company issued 8,452,500 shares of its common stock upon closing of its IPO for net proceeds of \$146.3 million and 510,524 shares of its common stock for an aggregate of \$249,701 in connection with the exercise of warrants.

**Exercise of Common Warrants**

During the year ended December 31, 2015, the Company issued 355,361 shares of its common stock to the Texas Treasury Safekeeping Trust Company (a transferee of the Office of the Governor - Economic Development and Tourism), pursuant to the cashless exercise provision of a warrant to purchase shares of the Company's common stock issued to the State of Texas on September 27, 2007. The Company did not receive any cash or other consideration.

**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 IPO on December 23, 2014, the underwriters exercised their over-allotment option to purchase 1,102,500 additional shares of the Company's common stock at a price to the public 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 connection with the restructuring of the license agreement with ARIAD Pharmaceuticals, Inc. (ARIAD), the Company repurchased from ARIAD 677,463 shares of its common stock valued at approximately \$5.1 million. See Note 11 to the financial statements included herein.

**Preferred Stock**

Upon the closing of the IPO on December 17, 2014, all outstanding convertible preferred stock was converted into 16,230,777 shares of common stock on a one-to-one basis. No convertible preferred stock was outstanding as of December 31, 2016 and 2015.

**NOTE 9 - 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.

Table of Contents

## Notes to the Financial Statements

## 2006 Stock Option Plan

The 2006 Stock Option Plan (the 2006 Plan) provided for the issuance of non-qualified stock options to employees, including officers, non-employee directors and consultants to the Company. A total of 146,210 and 151,410 options were outstanding under this plan as of December 31, 2016 and 2015. As of December 31, 2016, there were no additional shares available for grant under the 2006 Plan. During 2016 and 2015, a total of 5,200 and 15,646 options, respectively, were exercised for cash proceeds to the company of \$2,652 and \$6,980, respectively.

## 2011 Stock Option Plan

The 2011 Stock Option Plan (the 2011 Plan) provided for the issuance of incentive and non-qualified stock options to employees, including officers, non-employee directors and consultants to the Company. The 2011 Plan replaced the 2006 Plan. There were 2,051,413 and 2,256,120 outstanding options under this plan at December 31, 2016 and 2015, respectively. As of December 31, 2016, there were no additional shares available for grant under this plan. During 2016 and 2015, a total of 179,002 and 166,592 options, respectively, were exercised for cash proceeds to the company of \$0.7 million and \$0.5 million, respectively.

## 2014 Equity Incentive Plan

The 2014 Equity Incentive Plan (the 2014 Plan) became effective in December 2014, upon the closing of the IPO. The 2014 Plan provides for the issuance of equity awards, including incentive and non-qualified stock options and restricted stock awards to employees, including officers, non-employee directors and consultants to the Company or its affiliates. The 2014 Plan also provides for the grant of performance cash awards and performance-based stock awards. The aggregate number of shares of common stock that are authorized for issuance under the 2014 Plan is 2,990,354 shares, plus any shares subject to outstanding options that were granted under the 2011 Plan or 2006 Plan that are forfeited, terminated, expired or are otherwise not issued. There were 2,334,497 and 1,221,443 outstanding options under this plan at December 31, 2016 and 2015, respectively. During 2016, a total of 5,853 options were exercised for cash proceeds to the company of \$0.1 million. No shares were exercised for cash proceeds in 2015. There were 58,825 and 88,236 shares of restricted stock outstanding under the Plan at December 31, 2016 and 2015, respectively. As of December 31, 2016, there were 560,911 shares remaining to be issued.

## 2014 Employee Stock Purchase Plan

The 2014 Employee Stock Purchase Plan (the ESPP) provides for eligible Company employees, as defined by the ESPP, to be given an opportunity to purchase the Company's common stock at a discount, through payroll deductions, with stock purchases being made upon defined purchase dates. The ESPP authorizes the issuance of up to 550,000 shares of the Company's common stock to participating employees, and allows eligible employees to purchase shares of common stock at a 15% discount from the grant date fair market value. As of December 31, 2016, there were 494,681 shares remaining to be issued.

A summary of activity within the ESPP follows:

	Year Ended	
	December 31,	
	2016	2015
	(amounts in thousands)	
Deductions from employees	\$375	\$381
Share-based compensation expense recognized	\$244	\$242
Remaining share-based compensation expense	\$406	\$267
Proceeds received by the Company for ESPP	\$369	\$347

Weighted-average purchase price per common share \$10.97 \$16.01

Number of shares purchased by employees under ESPP 33,629 21,690

#### Share-Based Compensation Expense

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

Table of Contents

## Notes to the Financial Statements

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 fair value of the option grants have been estimated, with the following weighted-average assumptions:

	Year Ended December 31,					
	2016	2015	2014			
Risk-free interest rate	1.77 %	1.71 %	1.86 %			
Volatility	72 %	74 %	95 %			
Expected life (years)	6.08	6.08	6.09			
Expected dividend yield	0 %	0 %	0 %			

Share-based compensation for the years ended December 31, 2016, 2015 and 2014, are as follows:

	Year Ended December 31,		
	2016	2015	2014
	(in thousands)		
General and administrative	\$6,681	\$4,832	\$386
Research and development	\$5,656	3,577	525
Total	\$12,337	\$8,409	\$911

Stock option activity for the years ended December 31, 2016 and 2015 is as follows:

Options	Outstanding Stock Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term (in years)	(in thousands) Aggregate Intrinsic Value
Balance at December 31, 2014	2,733,793	\$ 5.09	8.39	\$ 49,076
Granted	1,089,767	\$ 22.23		
Exercised	(182,238)	\$ 2.64		
Forfeited	(12,349)	\$ 14.99		
Balance at December 31, 2015	3,628,973	\$ 10.32	8.03	\$ 39,021
Granted	1,159,957	\$ 17.43		
Exercised	(190,055)	\$ 4.07		\$ 2,448
Forfeited	(66,755)	\$ 12.46		
Balance at December 31, 2016	4,532,120	\$ 12.37	7.58	\$ 20,453
Exercisable as of December 31, 2016	2,302,155	\$ 8.22	6.59	\$ 17,095

Table of Contents

## Notes to the Financial Statements

Restricted stock share activity for the year ended December 31, 2016 and 2015 is as follows:

Restricted Stock Shares	Outstanding Restricted Shares	Weighted-Average Fair Value at Date of Grant Per Share
Balance at December 31, 2014	—	
Granted	117,647	\$ 19.00
Vested	(29,411)	\$ 19.00
Forfeited	—	
Balance at December 31, 2015	88,236	\$ 19.00
Granted	—	
Vested	(29,411)	\$ 19.00
Forfeited	—	
Balance at December 31, 2016	58,825	\$ 19.00

The following table includes share-based payment activity for the years ended December 31, 2016, 2015 and 2014:

	Year Ended December 31,		
	2016	2015	2014
	(in thousands, except per share)		
Weighted-average grant date fair value of options granted	\$11.24	\$16.09	\$13.30
Weighted-average grant date fair value of restricted shares granted	\$—	\$—	\$19.00
Aggregate intrinsic value of options exercised	\$2,448	\$3,236	\$59
Total fair value of restricted shares vested	\$607	\$656	\$—
Cash received by Company upon option exercises	\$774	\$482	\$11

The following table summarizes the options outstanding and exercisable at December 31, 2016:

Options Outstanding			Options Exercisable			
Exercise Price	Total Shares	Weighted- Average Remaining Contractual Term (in years)	Weighted-Average Exercise Price	Total Shares	Weighted- Average Remaining Contractual Term (in years)	Weighted-Average Exercise Price
\$ .51 to \$2.55	1,268,465	5.34	\$ 2.31	1,252,742	5.32	\$ 2.31
\$ 7.47 to \$19.85	2,337,638	8.54	\$ 13.46	616,076	8.02	\$ 9.60
\$ 20.09 to \$24.48	926,017	8.23	\$ 23.42	433,337	8.24	\$ 23.36
Total	4,532,120	7.58	Total	2,302,155	6.59	

At December 31, 2016, total compensation cost not yet recognized was \$27.4 million and the weighted average period over which this amount is expected to be recognized is 2.37 years. The aggregate fair value of options and restricted shares vesting in the years ended December 31, 2016, 2015 and 2014 was \$12.2 million, \$5.5 million and \$0.3 million, respectively.

NOTE 10 - 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. The Grant Contract terminated on June 30, 2014. The terms



Table of Contents

Notes to the Financial Statements

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, the Company incurred \$1.4 million of expenses under the Grant Contract. There were no expenses under the Grant Contract in 2015 and 2016.

On November 16, 2016, the Company received notice of a Product Development award totaling approximately \$16.9 million from the Cancer Prevention and Research Institute of Texas, CPRIT. Assuming successful contract negotiations and execution, the CPRIT award would fund a portion of a three-year global clinical program comprising clinical trials for adult and pediatric patients with high-risk and intermediate-risk acute myeloid leukemia. The proposed studies are designed to evaluate the benefit of BPX-501 and rimiducid in the context of in vivo and ex vivo T cell depleted haploidentical hematopoietic stem cell transplantation. The CPRIT oversight committee met in February 2017 and agreed to move forward with the proposed terms of the grant agreement. The Company is currently in the process of completing a new contract with CPRIT and expects to begin a clinical development program supported by the CPRIT funding in the second half of 2017.

NIH Grant

During 2016, 2015 and 2014, the Company was awarded \$0.3 million, \$0.3 million and \$0.3 million, respectively, under a grant from the National Institutes of Health (NIH). The awards cover the period from April 2013 through March 2017. The awards were made pursuant to the authority of 42 USC 241 42 CFR 52, and are subject to the requirements of the statute. Funds spent on the grant are reimbursed through monthly reimbursement requests.

As of December 31, 2016, 2015 and 2014, funds spent under the grant were \$0.4 million, \$0.3 million and \$0.3 million, respectively. As of December 31, 2016 and 2015, the Company had an outstanding grant receivable of \$30,000 and \$57,000, respectively for grant expenditures that were paid and not yet been reimbursed.

NOTE 11 - ARIAD RESTRUCTURING COSTS

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 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.

Table of Contents

Notes to the Financial Statements

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 12 - COMMITMENTS AND CONTINGENCIES

Operating Lease Agreements

The Company leases its office and manufacturing facilities under non-cancelable operating leases that expire January 31, 2020 and August 31, 2026, respectively. Rent expense for non-cancelable operating leases with scheduled rent increases is recognized on a straight-line basis over the terms of the leases. Improvement reimbursements from the landlord of \$2.5 million are being amortized on a straight-line basis into rent expense over the terms of the leases. The difference between required lease payments and rent expense has been recorded as deferred rent. Rent expense was \$1.8 million in 2016, \$1.2 million in 2015, and \$0.4 million in 2014. Deferred rent was \$2.1 million as of December 31, 2016 and \$ 1.1 million as of December 31, 2015.

Escrow agreement related to the operating lease dated May 2015

According to the escrow agreements in the operating leases, the Company agreed to deposit into escrow a total of approximately \$9.6 million, which represents 110% of the Company's remaining share of the estimated build-out costs. The funds were deposited into an escrow account in December 2016 and reported as restricted cash as of December 31, 2016.

Escrow agreement related to the First Amendment of the operating lease dated July 2016

The Company agreed to deposit into escrow a total of approximately \$1.4 million, which represents 110% of the Company's remaining share of the estimated build-out costs. The \$1.4 million was deposited into an escrow account in January 2017.

Capital Lease Agreements - Equipment

The Company entered into multiple office equipment leases during both 2016 and 2015, which expire in 2021. The office equipment leases are being accounted for as capital leases under FASB Topic ASC 840 - Leases. The present value of the minimum lease payments are greater than 90% or more than the fair market value of the leased equipment and the lease terms are 6 years or the remaining term of the lease.

Aggregate future minimum annual payments under operating and capital leases at December 31, 2016, are as follows:

Year	OperatingCapital	
	Leases	Leases
	(in thousands)	
2017	\$1,982	\$ 59
2018	2,033	59
2019	2,087	59
2020	1,124	59
2021	1,079	37
Thereafter	5,448	0
Total minimum rentals	\$13,753	\$ 273

Table of Contents

Notes to the Financial Statements

Co-Development and Co-Commercialization Agreement - Adaptimmune Therapeutics plc

On December 16, 2016, the Company entered into a Co-Development and Co-Commercialization Agreement with and Adaptimmune Therapeutics plc (Adaptimmune) in order to facilitate a staged collaboration to evaluate, develop and commercialize next generation T cell therapies. Under the Agreement, the parties agreed to evaluate the Company's GoTCR technology (inducible MyD88/CD40 co-stimulation, or iMC) with Adaptimmune's affinity-optimized SPEAR® T cells for the potential to create enhanced TCR product candidates. Depending on results of the preclinical proof-of-concept phase, the parties expect to progress to a two-target co-development and co-commercialization phase. To the extent necessary, and in furtherance of the parties' proof-of-concept and co-development efforts, the parties granted each other a royalty-free, non-transferable, non-exclusive license covering their respective technologies for purposes of facilitating such proof-of-concept and co-development efforts. In addition, as to covered therapies developed under the agreement, the parties granted each other a reciprocal exclusive license for the commercialization of such therapies. With respect to any joint commercialization of a covered therapy, the parties agreed to negotiate in good faith the commercially reasonable terms of a co-commercialization agreement. The parties also agreed that any such agreement shall provide for, among other things, equal sharing of the costs of any such joint commercialization and the calculation of profit shares as set forth in the Agreement. The Agreement will expire on a country-by-country basis once the parties cease commercialization of the T cell therapies covered by the Agreement, unless earlier terminated by either party for material breach, non-performance or cessation of development, bankruptcy/insolvency, or failure to progress to co-development phase. There were no expenses recognized under the Adaptimmune agreement for the year ended December 31, 2016.

Collaboration Agreement - OPBG

In October 2016, the Company entered into a collaboration agreement with and Ospedale Pediatrico Bambino Gesù (OPBG), pursuant to which the Company and OPBG agreed to collaborate on research projects and early stage clinical trials for the design and development of various T cell immunotherapies. As consideration for OPBG's performance of the research under the agreement and grant of certain licenses to the Company, the Company agreed to fund an aggregate of up to \$4.7 million in project costs payable to OPBG or certain third party service providers, as applicable, over the term of the research, estimated to be 4 years. With respect to any inventions arising from the research, OPBG agreed to grant the Company an exclusive license to any such inventions, the terms of which will be set forth in a separate agreement. In addition, OPBG granted the Company paid-up, worldwide co-exclusive licenses for non-commercial development of OPBG's CD19 and GD2 CAR-T technologies, as well as paid-up, worldwide exclusive licenses to commercialize its CD19 and GD2 CAR-T technologies, each to be governed by a separate agreement. The expenses recognized under the OPBG Collaboration Agreement was \$0.6 million for the year ended December 31, 2016.

Collaboration Agreement - Leiden

In May 2016, the Company and Academisch Ziekenhuis Leiden (Leiden) entered into a research collaboration agreement pursuant to which the Company will provide Leiden with financial support for research to discover and validate high-affinity TCR product candidates targeting several cancer-associated antigens. The Company agreed to pay Leiden an aggregate of EURO 2,547,415 in quarterly installments during the three-year term of the research, which will be recognized as services are incurred. During the year ended December 31, 2016, \$0.1 million of research

services were recognized. With respect to any inventions arising from the research that are relevant to or useful for any high affinity TCR that is studied in the research, Leiden granted the Company an exclusive option to obtain an exclusive, worldwide license to practice and exploit such inventions. The parties agreed to negotiate in good faith the commercially reasonable terms of each such license agreement entered into between the parties, based on terms similar to those set forth in the previously executed license agreement between the parties and those specified in the agreement. The expenses recognized under the Leiden license agreement were \$0.9 million and \$0.2 million for the years ended December 31, 2016 and 2015, respectively.

License Agreement - Baylor

In March 2016, the Company and Baylor College of Medicine (BCM) entered into two additional license agreements pursuant to which the Company obtained exclusive rights to technologies and patent rights owned by BCM. The Company paid BCM a nonrefundable license fee of \$0.1 million, and could incur additional payments upon the achievement of certain milestone events as set forth in the agreement. If the Company is successful in developing any of the licensed technologies, resulting sales would be subject to a royalty payment in the low single digits. The expenses recognized under the Baylor License Agreement was \$0.1 million for the year ended December 31, 2016.

License Agreement - Agensys, Inc.

Table of Contents

Notes to the Financial Statements

On December 10, 2015, the Company and Agensys, Inc. (Agensys), entered into a license agreement (the Agreement), pursuant to which (i) Agensys granted the Company, within the field of cell and gene therapy of diseases in humans, an exclusive, worldwide license and sublicense to its patent rights directed to prostate stem cell antigen 1 (“PSCA”) and related antibodies, and (ii) the Company granted Agensys a non-exclusive, fully paid license to the Company’s patents directed to inventions that were made by the Company in the course of developing the Company’s licensed products, solely for use with Agensys therapeutic products containing a soluble antibody that binds to PSCA or, to the extent not based upon Bellicum’s other proprietary technology, to non-therapeutic applications of antibodies not used within the field. As consideration for the rights granted to the Company under the Agreement, the Company agreed to pay to Agensys a non-refundable upfront fee of \$3,000,000, which is included in license fee expense. The Company is also required to make aggregate milestone payments to Agensys of up to (i) \$5,000,000 upon the first achievement of certain specified clinical milestones for its licensed products, (ii) \$50,000,000 upon the achievement of certain specified clinical milestones for each licensed product, and (iii) \$75,000,000 upon the achievement of certain sales milestones for each licensed product. The Agreement additionally provides that the Company will pay to Agensys a royalty that ranges from the mid to high single digits based on the level of annual net sales of licensed products by the Company, its affiliates or permitted sublicensees. The royalty payments are subject to reduction under specified circumstances. These milestone and royalty payments will be expensed as incurred. Under the Agreement, Agensys also was granted the option to obtain an exclusive license, on a product-by-product basis, from the Company to commercialize in Japan each licensed product developed under the Agreement that has completed a phase 2 clinical trial. As to each such licensed product, if Agensys or its affiliate, Astellas Pharma, Inc., exercises the option, the Agreement provides that the Company will be paid an option exercise fee of \$5,000,000. In addition, the Agreement provides that the Company will be paid a royalty that ranges from the mid to high single digits based on the level of annual net sales in Japan of each such licensed product. If the option is exercised, the aggregate milestone payments payable by the Company to Agensys, described above with respect to each licensed product, would be reduced by up to an aggregate of \$65,000,000 upon the achievement of certain specified clinical and sales milestones. The Agreement will terminate upon the expiration of the last royalty term for the products covered by the Agreement, which is the earlier of (i) the date of expiration or abandonment of the last valid claim within the licensed patent rights covering any licensed products under the Agreement, (ii) the expiration of regulatory exclusivity as to a licensed product, and (iii) 10 years after the first commercial sale of a licensed product. Either party may terminate the Agreement upon a material breach by the other party that remains uncured following 60 days after the date of written notice of such breach (or 30 days if such material breach is related to failure to make payment of amounts due under the Agreement) or upon certain insolvency events. In addition, Agensys may terminate the Agreement immediately upon written notice to the Company if the Company or any of its affiliates or permitted sublicensees commences an interference proceeding or challenges the validity or enforceability of any of Agensys’ patent rights. There were no expenses recognized under the Agensys License Agreement for the year ended December 31, 2016. For the year ended December 31, 2015, \$3.0 million of license expenses were recognized.

License Agreement - BioVec

On June 10, 2015, the Company and BioVec Pharma, Inc. (BioVec) entered into a license agreement (the BioVec Agreement) pursuant to which BioVec agreed to supply the Company with certain proprietary cell lines and granted to the Company a non-exclusive, worldwide license to certain of its patent rights and related know-how related to such proprietary cell lines. As consideration for the products supplied and rights granted to the Company under the BioVec Agreement, the Company agreed to pay to BioVec an upfront fee of \$100,000 within ten business days of the effective date of the BioVec Agreement and a fee of \$300,000 within ten business days of its receipt of the first release of GMP

lot of the products licensed under the BioVec Agreement. In addition, the Company agreed to pay to BioVec an annual fee of \$150,000, commencing 30 days following the first filing of an Investigational New Drug Application (an IND filing), or its foreign equivalent, for a product covered by the license; with such annual fees being creditable against any royalties payable by the Company to BioVec under the BioVec Agreement. The Company also is required to make a \$250,000 milestone payment to BioVec for each of the first three licensed products to enter into a clinical phase trial and one-time milestone payments of \$2,000,000 upon receipt of a registration granted by the Federal Drug Administration or European Medicines Agency on each of the Company's first three licensed products. The BioVec Agreement additionally provides that the Company will pay to BioVec a royalty in the low single digits on net sales of products covered by the BioVec Agreement. The Company may also grant sublicenses under the licensed patent rights and know-how to third parties for limited purposes related to the use, sale and other exploitation of the products licensed under the BioVec Agreement. The BioVec Agreement will continue until terminated. The BioVec Agreement may be terminated by the Company, in its sole discretion, at any time upon 90 days written notice to BioVec. Either party may terminate the BioVec Agreement in the event of a breach by the other party of any material provision of the BioVec Agreement that remains uncured on the date that is 60 days after written notice of such failure or upon certain insolvency events that remain uncured following the date that is 30 days after the date of written notice to a party regarding such insolvency event. The Company recognized expenses of \$0.1 million and \$0.5 million, respectively, in connection with the BioVec License Agreement for the year ended December 31, 2015 and 2016, respectively.



## Table of Contents

### Notes to the Financial Statements

#### License Agreement - Leiden

On April 23, 2015, the Company and Academisch Ziekenhuis Leiden, also acting under the name Leiden University Medical Centre (Leiden), entered into a license agreement (the Leiden Agreement), pursuant to which Leiden granted to the Company an exclusive, worldwide license to its patent rights covering high affinity T-cell receptors targeting preferentially-expressed antigen in melanoma, (PRAME) and POU2AF1 epitopes. The license granted under the Leiden Agreement is subject to certain restrictions and to Leiden's retained right to use the licensed patents solely for academic research and teaching purposes, including research collaborations by Leiden with academic, non-profit research third parties; provided that Leiden provides 30 days advance written notice to the Company of such academic research collaborations. As consideration for the rights granted to the Company under the Leiden Agreement, the Company agreed to pay to Leiden an aggregate of EUR 75,000 in upfront fees within 30 days of the effective date of the Leiden Agreement. In addition, the Company agreed to pay to Leiden, beginning on the eighth anniversary of the effective date of the Leiden Agreement, annual minimum royalty payments of EUR 30,000. The Company also is required to make milestone payments to Leiden of up to an aggregate of EUR 1,025,000 for each of the first licensed product that is specific to PRAME and to POU2AF1. The Leiden Agreement additionally provides that the Company will pay to Leiden a royalty in the low single digits on net sales of products covered by the Leiden Agreement. If the Company enters into a sublicensing agreement with a third party related to a product covered by the Leiden Agreement, the Company agreed to pay Leiden a percentage ranging in the low double digits on all non-royalty income received from sublicensing revenue directly attributable to the sublicense, dependent on whether the Company is in phase 1/2, phase 2 or phase 3 at the time that the Company enters into any such sublicensing agreement. Under the Leiden Agreement, the Company and Leiden entered into a sponsored research agreement, pursuant to which the Company is required to pay Leiden up to EUR 300,000 over a three years period during the term of the sponsored research agreement. The Leiden Agreement will expire upon the expiration of the last patent included in the licensed patent rights. The Leiden Agreement may be terminated earlier upon mutual written agreement between the Company and Leiden, and at any time by the Company upon six months written notice to Leiden. Leiden may terminate the Leiden Agreement in the event of a failure by the Company to pay any amounts due under the Leiden Agreement that remains uncured on the date that is 30 days after written notice of such failure. Either party may terminate the Leiden Agreement upon a material breach by the other party that remains uncured following 30 days after the date of written notice of such breach or upon certain insolvency events that remain uncured following the date that is 45 days after the date of written notice to a party of such insolvency event. The Company recognized \$84,000 of expenses in connection with the Leiden License Agreement for the year ended December 31, 2015. The Company was not required to make any milestone payments for the year ended December 31, 2015.

#### Employment agreements

The Company has signed agreements with thirteen 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 6 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 \$4.9 million at the rate of compensation in effect at December 31, 2016. In addition, the agreements provide for continuation of certain insurance and other benefits for periods of 6 to 18 months.

#### 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.



Table of Contents

## Notes to the Financial Statements

## NOTE 13 - INCOME TAXES

The Company did not recognize tax expense during 2016, 2015 or 2014.

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,		
	2016	2015	2014
	(in thousands)		
U.S. tax benefit at statutory rate	\$(23,542)	\$(16,506)	\$(28,548)
Meals and entertainment	22	24	10
Stock options	394	12	98
Warrant expense	—	—	8,286
Federal deferred tax true-up	32	(187)	) —
Return to provision	—	(2)	) —
Deferred tax valuation allowances	24,872	17,920	20,586
Research and development credit	(1,778)	) (1,261)	) (432)
Income tax expense	\$—	\$—	\$—

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, 2016 and 2015 are as follows:

	December 31,	
	2016	2015
	(in thousands)	
Deferred tax liabilities:		
Unrealized gain on investment securities	\$ (6 )	\$ —
Depreciation	(4 )	(933)
Total deferred tax liabilities	(10 )	(933)
Deferred tax assets:		
Net operating loss carryforward	48,152	28,229
Nonqualified stock options	5,126	2,248
Restricted stock expense	20	20
Employee stock purchase plan	—	82
Tenant improvement liability	645	341
Intangible assets	14,920	15,716
Unrealized loss on investment securities	—	103
Research and development credit	4,296	2,519
Other	67	23
Total deferred tax assets	73,226	49,281
Valuation allowance	(73,216)	(48,348)
Total deferred tax	\$ —	\$ —
Net current deferred tax liability	\$ —	\$ —
Net non-current deferred tax asset	—	—

As of December 31, 2016 and 2015, the Company had gross federal income tax net operating loss (NOL) carryforwards of \$142.2 million and \$83.0 million, respectively, and federal research tax credits of \$4.3 million and \$2.5 million, respectively. The NOL carryforwards will expire beginning in 2025, 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.

Table of Contents

## Notes to the Financial Statements

Due to the uncertainty surrounding the realization of the benefits of its deferred assets, including NOL carryforwards, the Company has provided a 100% valuation allowance on its deferred tax assets at December 31, 2016 and 2015. The increases in the valuation allowance were \$24.9 million and \$18.0 million for the years ended December 31, 2016 and 2015, respectively.

The Internal Revenue Code Section 382 limits NOL 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 NOL and tax credit carryforwards may be significantly restricted.

## NOTE 14 - SELECTED QUARTERLY FINANCIAL DATA (UNAUDITED)

Selected quarterly financial data (unaudited) for the year ended December 31, 2016 and 2015 is presented below:

	(in thousands except per share data)			
	First Quarter	Second Quarter	Third Quarter	Fourth Quarter
2016				
Total revenues	\$92	\$101	\$114	\$81
Loss from operations	\$(15,180)	\$(16,259)	\$(17,428)	\$(19,513)
Net loss	\$(15,075)	\$(16,509)	\$(17,719)	\$(19,938)
Net loss per share attributable to common shareholders - basic and diluted	\$(0.56 )	\$(0.61 )	\$(0.66 )	\$(0.74 )
2015				
	First Quarter	Second Quarter	Third Quarter	Fourth Quarter (1)
Total revenues	\$107	\$84	\$57	\$34
Loss from operations	\$(7,808 )	\$(10,705)	\$(13,617)	\$(17,005)
Net loss	\$(7,758 )	\$(10,534)	\$(13,408)	\$(16,848)
Net loss per share attributable to common shareholders - basic and diluted	\$(0.30 )	\$(0.40 )	\$(0.51 )	\$(0.63 )

(1) The 2015 fourth quarter results include a non-refundable upfront fee to Agensys of \$3.0 million under the license agreement. See Note 12 to the financial statements included herein.

## NOTE 15 - SUBSEQUENT EVENTS

Effective January 30, 2017, Thomas J. Farrell, resigned from his position as the President and Chief Executive Officer of the Company. In connection with Mr. Farrell's resignation, effective January 30, 2017 the Board of Directors of the Company, appointed Richard A. Fair to serve as the Company's President and Chief Executive Officer.

On March 8, 2017, the Company borrowed an additional \$10.0 million under its Loan Agreement with Hercules. The Company now has total outstanding principal under the Loan Agreement of approximately \$30.0 million and an additional end of term commitment of \$695,000 and a facility charge of \$75,000. In addition, the interest only period was extended for another six months.

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

Our management, with the participation of our Chief Executive Officer and Chief Financial and Accounting Officer (our principal executive officer and principal financial officer, respectively), evaluated the effectiveness of our disclosure controls and procedures, as

## Table of Contents

defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, as of December 31, 2016. The term “disclosure controls and procedures,” as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits 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 a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company’s management, including its principal executive and principal financial officers, as appropriate, to allow timely decisions regarding required disclosure.

Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of December 31, 2016, our Chief Executive Officer and Chief Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective.

### Management’s Annual Report on Internal Controls over Financial Reporting

Management is responsible for establishing and maintaining adequate internal control over financial reporting, as such term is defined in Exchange Act Rule 13a-15(f). Our internal control over financial reporting is designed under the supervision of our Chief Executive Officer and Chief Financial Officer to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with United States generally accepted accounting principles.

Management, including our Chief Executive Officer and Chief Financial and Accounting Officer, has assessed the effectiveness of our internal control over financial reporting based on the framework set forth by the Committee of Sponsoring Organizations of the Treadway Commission in Internal Control-Integrated Framework. Based on our evaluation, management has concluded that our internal control over financial reporting was effective as of December 31, 2016.

This Annual Report does not include an attestation report of our registered public accounting firm due to a transition period established by the JOBS Act applicable to emerging growth companies.

### Inherent Limitations of Internal Controls

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risks that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

### Changes in Internal Control over Financial Reporting

There were no changes in our internal control over financial reporting during our latest fiscal quarter that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

## ITEM 9B. Other Information

On March 8, 2017, we borrowed an additional \$10.0 million under our loan and security agreement with Hercules. As previously reported, we entered into the loan agreement and initially borrowed \$15.0 million on March 10, 2016 and we subsequently borrowed an additional \$5.0 million on September 15, 2016, as described in our Current Reports on Form 8-K filed with the SEC on March 11, 2016, or the Prior 8-K, and September 20, 2016, respectively. We now have total outstanding principal under the loan agreement of approximately \$30.0 million.

Additional detail regarding the loan agreement is contained in Item 1.01 of the Prior 8-K and is incorporated herein by reference. The descriptions of the loan agreement contained in the Prior 8-K and herein are qualified in their entirety by reference to the complete text of the loan agreement, including the exhibits thereto, a copy of which is filed as Exhibit 10.1 to our Quarterly Report on Form 10-Q filed with the SEC on May 9, 2016 and included on the Exhibit Index to this Annual Report.





Table of Contents

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 headed “Election of Directors,” “Executive Officers” and “Section 16(a) Beneficial Ownership Reporting Compliance” in our definitive proxy statement for our 2017 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, 2016, 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 and accounting officer or controller, or persons performing similar functions, known as the Code of Business Conduct and Ethics. The Code of Business Conduct and Ethics is available on our website at [www.bellicum.com](http://www.bellicum.com) under the Corporate Governance section of our Investors & Media page. 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 headed “Executive and Director 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 “Equity Benefit Plans” and “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 and Director 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 headed “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 headed “Principal Accounting Fees and Services” in our Proxy Statement and is incorporated herein by reference.

Table of Contents

PART IV

ITEM 15. Exhibits, Financial Statements and Schedules

(a)(1) Financial Statements.

The response to this portion of Item 15 is set forth under Part II, Item 8 above.

(a)(2) Financial Statement Schedules.

None.

(a)(3) Exhibits.

See the Exhibit Index immediately following the signature page of this Annual Report. The exhibits listed in the Exhibit Index below are filed or incorporated by reference as part of this Annual Report.

Table of Contents

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 13, 2017 By: /s/ Richard A. Fair

Richard A. Fair

President and Chief Executive Officer

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints Richard A. Fair 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/ Richard A. Fair Richard A. Fair	President, Chief Executive Officer and Member of the Board of Directors (Principal Executive Officer)	March 13, 2017
/s/ Alan A. Musso Alan A. Musso	Chief Financial Officer and Treasurer (Principal Financial and Accounting Officer)	March 13, 2017
/s/ James Brown James Brown	Chairman of the Board of Directors	March 13, 2017
/s/ James M. Daly James M. Daly	Member of the Board of Directors	March 13, 2017
/s/ Stephen R. Davis Stephen R. Davis	Member of the Board of Directors	March 13, 2017
/s/ Reid M. Huber, Ph.D. Reid M. Huber, Ph.D.	Member of the Board of Directors	March 13, 2017
/s/ Frank B. McGuyer Frank B. McGuyer	Member of the Board of Directors	March 13, 2017
/s/ Kevin M. Slawin, M.D. Kevin M. Slawin, M.D.	Member of the Board of Directors	March 13, 2017
/s/ Jon P. Stonehouse Jon P. Stonehouse	Member of the Board of Directors	March 13, 2017



Table of Contents

INDEX TO EXHIBITS

Exhibit  
Description  
Number

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 Registration Rights Agreement by and among the Registrant and Baker Brothers Life Sciences, LP, and two of its affiliated funds, dated January 15, 2016.

10.1 Form of Indemnification Agreement by and between the Registrant and its directors and officers (incorporated by reference to Exhibit 10.1 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).

10.2 Bellicum Pharmaceuticals, Inc. 2006 Stock Option Plan and Form of Nonqualified Stock Option Agreement (incorporated by reference to Exhibit 10.2 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).

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

10.4 Bellicum Pharmaceuticals, Inc. 2014 Equity Incentive Plan and Forms of Stock Option Grant Notices, Stock Option Agreements and Notices of Exercise, Form of Restricted Stock Award Notice and Restricted Stock Award Agreement, Form of Restricted Stock Unit Grant Notice and Restricted Stock Unit Award Agreement thereunder.

10.5 Bellicum Pharmaceuticals, Inc. Non-Employee Director Compensation Policy.

10.6 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).

10.7 Amended and Restated Employment Agreement by and between the Registrant and David M. Spencer, Ph.D., dated November 17, 2014 (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).

Employment Agreement by and between the Registrant and Joseph H. Senesac, dated November 16, 2014  
~~10.8~~ 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).

Employment Agreement by and between the Registrant and Peter L. Hoang, dated November 17, 2014 (incorporated  
~~10.9~~ 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).

Notice of Expansion of Licensed Field to Obtain Additional Exclusive Rights (incorporated by reference to  
~~10.10~~ 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).

Amended and Restated License Agreement by and between the Registrant and ARIAD Pharmaceuticals, Inc., dated  
~~10.11~~ 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).

Omnibus Amendment Agreement by and between Registrant and ARIAD Pharmaceuticals, Inc., dated October 3,  
~~10.12~~ 2012\* (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).

Table of Contents

Exhibit  
Description  
Number

Exclusive License Agreement by and between the Registrant and Baylor College of Medicine, dated March 20, 2008  
10.13\* (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).

Exclusive License Agreement by and between the Registrant and Baylor College of Medicine, dated June 27, 2010  
10.14\* (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).

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).

Exclusive License Agreement by and between the Registrant and Baylor College of Medicine, effective November 1, 2016\* (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).

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).

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).

Second Amendment to Lease Agreement by and between Registrant and Sheridan Hills Developments L.P., dated November 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).

Third Amendment to Lease Agreement by and between Registrant and Sheridan Hills Developments L.P., dated July 12, 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).

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).

Loan and Security Agreement by and between the Registrant and Comerica Bank, dated December 13, 2012  
10.22\* (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).

First Amendment to Loan and Security Agreement by and between the Registrant and Comerica Bank, dated March 10, 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).

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).

10.25+ 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).

10.26+ Incentive Award Program (incorporated by reference to Exhibit 10.1 to the Registrant's report on Form 8-K filed with the SEC on February 27, 2015).

10.27+ Amended and Restated Employment Agreement by and between the Registrant and Annemarie Moseley, Ph.D., dated April 1, 2015 (incorporated by reference to Exhibit 10.1 to the Registrant's report on Form 8-K filed with the SEC on April 7, 2015).

10.28+ Employment Agreement by and between the Registrant and Kevin M. Slawin, M.D., Dated April 6, 2015 (incorporated by reference to Exhibit 10.2 to the Registrant's Registration report on Form 8-K filed with the SEC on April 7, 2015).

10.29+ Employment Agreement by and between the Registrant and Ken Moseley, dated April 1, 2015 (incorporated by reference to Exhibit 10.3 to the Registrant's report on Form 10-Q filed with the SEC on May 12, 2015).

10.30+ License Agreement by and between the Registrant and Academish Ziekenhuis Leiden, also acting under the name Leiden University Medical Centre, effective as of April 20, 2015 (incorporated by reference to Exhibit 10.1 to the Registrant's report on Form 10-Q filed with the SEC on August 13, 2015).



Table of Contents

Exhibit  
Description  
Number

~~10.31~~ License Agreement by and between the Registrant and BioVec Pharma, Inc., dated as of June 4, 2015 (incorporated by reference to Exhibit 10.2 to the Registrant's report on Form 10-Q filed with the SEC on August 13, 2015).

~~10.32~~ Agreement by and between the Registrant and Sheridan Hills Developments L.P., dated as of May 6, 2015.

~~10.33~~ Exclusive License Agreement by and between the Registrant and Agensys, Inc., effective as of December 10, 2015.

~~10.34~~ Consulting Agreement by and between the Registrant and Kevin M. Slawin, M.D., effective as of May 18, 2016 (incorporated by reference to Exhibit 10.1 to the Registrant's report on Form 10-Q filed with the SEC on August 8, 2016).

~~10.35~~ Loan and Security Agreement by and between the Registrant and Hercules Capital, Inc., dated as of March 10, 2016 (incorporated by reference to Exhibit 10.1 to the Registrant's report on Form 10-Q filed with the SEC on May 9, 2016).

~~10.36~~ Sponsored Research Agreement No. 2 by and between the Registrant and Academish Ziekenhuis Leiden, also acting in the name Leiden University Medical Centre, effective as of May 20, 2016 (incorporated by reference to Exhibit 10.2 to the Registrant's report on Form 10-Q filed with the SEC on August 8, 2016).

~~10.37~~ First Amendment to Lease Agreement by and between the Registrant and Life Science Plaza Investment Group, LP, effective as of July 11, 2016 (incorporated by reference to Exhibit 10.3 to the Registrant's report on Form 10-Q filed with the SEC on August 8, 2016).

~~10.38~~ Fifth Amendment to Lease Agreement by and between the Registrant and Sheridan Hills Developments L.P., effective as of September 24, 2015 (incorporated by reference to Exhibit 10.1 to the Registrant's report on Form 10-Q filed with the SEC on November 9, 2016).

~~10.39~~ Second Amendment to Lease Agreement by and between the Registrant and Life Science Plaza Investment Group, LP, effective as of September 26, 2016 (incorporated by reference to Exhibit 10.3 to the Registrant's report on Form 10-Q filed with the SEC on November 9, 2016).

~~10.40~~ Research Collaboration Agreement by and between the Registrant and Ospedale Pediatrico Bambino Gesù, effective as of October 28, 2016.

~~10.41~~ Co-Development and Co-Commercialisation Agreement by and between the Registrant and Adaptimmune Limited, effective as of December 16, 2016.

~~10.42~~ Agreement by and between the Registrant and Thomas J. Farrell, dated January 25, 2017.

~~10.43~~ Agreement by and between the Registrant and Richard A. Fair, dated January 25, 2017.

~~23~~ Consent of Ernst & Young LLP, an Independent Registered Public Accounting Firm.

~~24~~ Power of Attorney. Reference is made to the signature page hereto.

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.

# Certain provisions of this exhibit have been omitted pursuant to a request for confidential treatment.