

BELLICUM PHARMACEUTICALS, INC  
Form 10-Q  
August 08, 2017  
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UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended June 30, 2017

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 2836 20-1450200  
(State or other jurisdiction of (Primary Standard Industrial (I.R.S. Employer  
incorporation or organization) Classification Code Number) Identification Number)

2130 W. Holcombe Blvd., Ste. 800

Houston, TX 77030

(832) 384-1100

(Address, including zip code, and telephone number, including  
area code, of registrant's principal executive offices)

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

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

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated

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filer,” “smaller reporting company,” and “emerging growth company” in Rule 12b-2 of the Exchange Act. (Check one):

Large accelerated filer  Accelerated filer   
Non-accelerated filer  (Do not check if a smaller reporting company) Smaller reporting company   
Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act).

Yes  No

As of July 28, 2017, there were 33,226,519 outstanding shares of Bellicum’s common stock, par value, \$0.01 per share.

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## PART I. FINANCIAL INFORMATION

## Item 1. Financial Statements

Bellicum Pharmaceuticals, Inc.  
 Condensed Consolidated Balance Sheets  
 (In thousands, except share and par value amounts)

	June 30, 2017 (Unaudited)	December 31, 2016
<b>ASSETS</b>		
Current assets:		
Cash and cash equivalents	\$ 75,148	\$ 33,140
Investment securities, available for sale - short-term	55,081	70,632
Accounts receivable, interest and other receivables	275	334
Prepaid expenses and other current assets	2,621	1,504
Total current assets	133,125	105,610
Investment securities, available for sale - long-term	4,405	—
Property and equipment, net	25,458	16,504
Restricted cash	4,383	9,640
Other assets	353	283
<b>TOTAL ASSETS</b>	<b>\$ 167,724</b>	<b>\$ 132,037</b>
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Current liabilities:		
Accounts payable	\$ 2,103	\$ 3,623
Accrued expenses and other current liabilities	10,164	9,363
Current maturities of long-term debt	3,412	1,787
Current portion of capital lease obligations	27	21
Current portion of deferred rent	256	319
Total current liabilities	15,962	15,113
Long-term liabilities:		
Long-term debt	27,116	18,436
Capital lease obligation	148	141
Deferred rent	1,791	1,773
<b>TOTAL LIABILITIES</b>	<b>45,017</b>	<b>35,463</b>
Commitments and contingencies:		
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 June 30, 2017 and December 31, 2016, 33,870,692 shares issued and 33,193,229 shares outstanding at June 30, 2017; 27,833,028 shares issued and 27,155,565 shares outstanding at December 31, 2016	339	278
Treasury stock: 677,463 shares held at June 30, 2017 and December 31, 2016	(5,056 )	(5,056 )
Additional paid-in capital	404,593	332,068
Accumulated other comprehensive income (loss)	(6 )	17 )
Accumulated deficit	(277,163 )	(230,733 )
Total stockholders' equity	122,707	96,574
<b>TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY</b>	<b>\$ 167,724</b>	<b>\$ 132,037</b>

See accompanying notes, which are an integral part of these unaudited consolidated financial statements.

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Bellicum Pharmaceuticals, Inc.

Condensed Consolidated Statements of Operations and Comprehensive Income (Loss)

(In thousands, except share and per share amounts)

(Unaudited)

	Three months ended June 30,		Six months ended June 30,	
	2017	2016	2017	2016
<b>REVENUES</b>				
Grants	\$—	\$ 101	\$ 128	\$ 193
Total revenues	—	101	128	193
<b>OPERATING EXPENSES</b>				
Research and development	17,959	12,031	33,254	22,889
License fees	343	150	698	280
General and administrative	5,486	4,179	11,413	8,463
Total operating expenses	23,788	16,360	45,365	31,632
Loss from operations	(23,788 )	(16,259 )	(45,237 )	(31,439 )
<b>OTHER INCOME (EXPENSE):</b>				
Interest income	307	236	504	463
Interest expense	(976 )	(486 )	(1,697 )	(608 )
Total other expense	(669 )	(250 )	(1,193 )	(145 )
<b>NET LOSS</b>	<b>\$(24,457)</b>	<b>\$(16,509)</b>	<b>\$(46,430)</b>	<b>\$(31,584)</b>
Net loss per common share attributable to common shareholders, basic and diluted	\$(0.74 )	\$(0.61 )	\$(1.54 )	\$(1.17 )
Weighted-average shares outstanding, basic and diluted	33,074,463	26,910,284	30,201,116	26,896,405
Net loss	\$(24,457)	\$(16,509)	\$(46,430)	\$(31,584)
<b>Other comprehensive income (loss):</b>				
Unrealized gain (loss) on investment securities	(16 )	152	(23 )	398
Comprehensive loss	\$(24,473)	\$(16,357)	\$(46,453)	\$(31,186)

See accompanying notes, which are an integral part of these unaudited consolidated financial statements.

Bellicum Pharmaceuticals, Inc.  
Condensed Consolidated Statements of Cash Flows  
(In thousands)  
(Unaudited)

	Six months ended June 30,	
	2017	2016
<b>CASH FLOWS FROM OPERATING ACTIVITIES:</b>		
Net loss	\$(46,430)	\$(31,584)
Adjustments to reconcile net loss to net cash used in operating activities:		
Share-based compensation	6,554	6,183
Depreciation expense	1,648	951
Amortization of premium on investment securities, net	123	340
Amortization of lease liability	(45)	(85)
Amortization of deferred financing costs	380	150
Loss on disposition of fixed assets	—	20
Changes in operating assets and liabilities:		
Receivables	59	58
Prepaid expenses and other assets	(1,187)	256
Accounts payable	(1,587)	(1,126)
Accrued liabilities and other	(2,138)	287
<b>NET CASH USED IN OPERATING ACTIVITIES</b>	<b>(42,623)</b>	<b>(24,550)</b>
<b>CASH FLOWS FROM INVESTING ACTIVITIES:</b>		
Purchases of investment securities	(28,020)	(22,700)
Proceeds from sale of investment securities	39,020	15,858
Purchases of property and equipment	(7,573)	(4,567)
<b>CASH PROVIDED BY (USED IN) INVESTING ACTIVITIES</b>	<b>3,427</b>	<b>(11,409)</b>
<b>CASH FLOWS FROM FINANCING ACTIVITIES:</b>		
Proceeds from stock offering, net of offering costs	64,568	—
Proceeds from issuance of common stock - ESPP	167	188
Proceeds from exercise of stock options	1,297	285
Proceeds from notes payable	10,000	15,000
Payment of debt issuance costs	(75)	(199)
Payment on capital lease obligation	(10)	(7)
<b>NET CASH PROVIDED BY FINANCING ACTIVITIES</b>	<b>75,947</b>	<b>15,267</b>
<b>NET CHANGE IN CASH, CASH EQUIVALENTS AND RESTRICTED CASH</b>	<b>36,751</b>	<b>(20,692)</b>
<b>CASH, CASH EQUIVALENTS AND RESTRICTED CASH AT BEGINNING OF PERIOD</b>	<b>42,780</b>	<b>70,241</b>
<b>CASH, CASH EQUIVALENTS AND RESTRICTED CASH AT END OF PERIOD</b>	<b>\$79,531</b>	<b>\$49,549</b>
<b>SUPPLEMENTAL CASH FLOW INFORMATION:</b>		
<b>NON-CASH INVESTING AND FINANCING ACTIVITIES:</b>		
Purchases of equipment in accounts payables and accrued liabilities	\$3,006	\$1,119
Accrued debt issuance costs	\$695	\$1,216
Capital lease obligations incurred for equipment	\$23	\$34

See accompanying notes, which are an integral part of these unaudited consolidated financial statements.



Bellicum Pharmaceuticals, Inc.

Notes to Unaudited Condensed Consolidated Financial Statements

#### NOTE 1 - ORGANIZATION AND BUSINESS DESCRIPTION

Bellicum Pharmaceuticals, Inc., or Bellicum, was incorporated in Delaware in July 2004 and is based in Houston, Texas. Bellicum 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. Bellicum 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.

In January 2017, Bellicum formed a wholly-owned subsidiary, Bellicum Pharma Limited, a private limited company organized under the laws of the United Kingdom for the purpose of developing product candidates in Europe. Bellicum and Bellicum Pharma Limited are collectively referred to herein as the Company.

#### NOTE 2 - BASIS OF PRESENTATION AND MANAGEMENT PLANS

The accompanying interim consolidated financial statements are unaudited. These unaudited interim financial statements have been prepared in accordance with U.S. generally accepted accounting principles (GAAP) and follow the requirements of the U.S. Securities and Exchange Commission (SEC) for interim reporting. As permitted under those rules, certain footnotes or other financial information that are normally required by GAAP have been omitted. In management's opinion, the unaudited interim condensed consolidated financial statements have been prepared on the same basis as the audited financial statements and include all adjustments necessary for the fair presentation of the Company's financial position and its results of operations and its cash flows for the periods presented. All such adjustments are normal and recurring in nature. These statements should be read in conjunction with the Company's Annual Report on Form 10-K filed for the fiscal year ended December 31, 2016 (the Annual Report). A copy of the Annual Report is available on the SEC's website, [www.sec.gov](http://www.sec.gov), under the Company's ticker symbol "BLCM" or on Bellicum's website, [www.bellicum.com](http://www.bellicum.com). The results for the interim periods are not necessarily indicative of the results expected for the full fiscal year or any other interim period. Any reference in these footnotes to applicable guidance is meant to refer to GAAP as found in the Accounting Standards Codification (ASC) and Accounting Standards Update (ASU) of the Financial Accounting Standards Board (FASB).

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 June 30, 2017, the Company had an accumulated deficit of \$277.2 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 3 - SIGNIFICANT ACCOUNTING POLICIES

##### Use of Estimates

The preparation of the consolidated 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.

Consolidation

All financial information presented includes the accounts of the Company and its wholly-owned subsidiary, for which there has been no activity to date. All significant intercompany balances and transactions have been eliminated in consolidation.

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### Reclassifications

Certain prior period amounts in research and development expenses pertaining to license fees have been reclassified and listed separately to conform to the current period presentation. The reclassifications did not impact net loss or net loss per share.

### Revenue Recognition

The Company has not yet generated any revenue from product sales. The Company's source of revenue for 2017 and 2016 has been grant revenue related to a \$1.3 million research grant from the National Institutes of Health (NIH) covering the period from April 2013 to March 2017. The grant contract with the NIH expired at the end of the first quarter. The Company accrued revenue as work was performed and qualifying costs were incurred.

### 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.

### 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 shares of common stock outstanding during the period without consideration for common stock equivalents.

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

	As of June 30,	
	2017	2016
Common Stock Equivalents:	Number of shares	
Options to purchase common stock	4,929,137	4,518,961
Unvested shares of restricted stock units	81,250	—
Unvested shares of restricted stock	44,119	88,236
Total common stock equivalents	5,054,506	4,607,197

### 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 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

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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

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.

#### 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 accrued rent. Any lease incentives received are deferred and amortized over the term of the lease.

#### 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.

#### Contract Manufacturing Services

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

#### Share-Based Compensation

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

#### Application of New Accounting Standards

During 2017, the Company adopted ASU No. 2016-09, "Compensation - Stock Compensation (Topic 718): Improvements to Employee Share-Based Payment Accounting", which is intended to simplify the financial reporting of the income tax impacts of share-based compensation arrangements. The classification guidance under ASU No. 2016-09 requires the recognition of excess tax benefits from share-based compensation arrangements as a discrete item within income tax benefit rather than additional paid-in capital and the classification guidance requiring presentation of excess tax benefits from share-based compensation arrangements as an operating activity on the statement of cash flows, rather than as a financing activity.

The adoption of ASU No 2016-09 had no immediate impact on our financial statements and related disclosures because the Company does not currently recognize a tax benefit related to share-based compensation expense as we maintain net operating loss carryforwards and have established a valuation allowance against the entire net deferred tax asset as of June 30, 2017. Further, we have elected to continue to estimate the number of stock-based awards expected to vest, as permitted by ASU 2016-09, rather than electing to account for forfeitures as they occur.

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 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 adopted this standard in 2017.

#### 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 consolidated 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 consolidated financial statements.

**NOTE 4 - CASH, CASH EQUIVALENTS AND RESTRICTED CASH**

As of June 30, 2017 and December 31, 2016, respectively, the Company maintained \$4.4 million and \$9.6 million as restricted cash. The funds are being held by an escrow agent to cover the construction costs related to the Company's facility lease. The restricted cash is subject to the terms of the escrow agreement and the requirements specified therein. The amount will decrease as the Company and its landlord authorize completion of certain aspects of the building improvements.

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.

	June 30, December 2017 31, 2016 (in thousands)	
Cash and cash equivalents <sup>(1)</sup>	\$75,148	\$ 33,140
Restricted cash, noncurrent	4,383	9,640
Total cash, cash equivalents and restricted cash shown in the statements of cash flows	\$79,531	\$ 42,780

<sup>(1)</sup> As of June 30, 2017 and December 31, 2016, the Company invested approximately \$65.1 million and \$23.5 million, respectively, in cash equivalent instruments.

**NOTE 5 - FAIR VALUE MEASUREMENTS AND INVESTMENT SECURITIES****Fair Value Measurement**

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 June 30, 2017 and December 31, 2016, respectively:

	Fair Value Measurements at Reporting Date			
	Balance at June 30, 2017	Quoted prices in active markets for identical assets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
(in thousands)				
Cash Equivalents:				
Money market funds	\$65,060	\$65,060	\$ —	\$ —
Total Cash Equivalents	\$65,060	\$65,060	\$ —	\$ —
Investment Securities:				
U.S. government agency-backed securities	\$19,120	\$ —	\$ 19,120	\$ —
Corporate debt securities	38,710	—	38,710	—
Municipal bonds	1,656	—	1,656	—
Total Investment Securities	\$59,486	\$ —	\$ 59,486	\$ —

	Fair Value Measurements at Reporting Date			
	Balance at December 31, 2016	Quoted prices in active markets for identical assets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
(in thousands)				
Cash Equivalents:				
Money market funds	\$23,459	\$23,459	\$ —	\$ —
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	\$ —

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.

Investment securities, all classified as available-for-sale, consisted of the following as of June 30, 2017 and December 31, 2016:

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Aggregate Estimated Fair Value
(in thousands)				
June 30, 2017				
Investment Securities:				
U.S. government agency-backed securities	\$19,145	\$ —	\$ (25 )	\$ 19,120

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Corporate debt securities	38,691	37	(18	)	38,710
Municipal bonds	1,656	—	—		1,656
Total Investment Securities	\$59,492	\$ 37	\$ (43	)	\$ 59,486

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	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

The Company's investment securities as of June 30, 2017, will reach maturity between July 2017 and January 2019, with a weighted-average maturity date in February 2018.

At December 31, 2016, the Company 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 were considered available for use in current operations. However, as of June 30, 2017, the Company reclassified the investment securities with maturity dates beyond one year as non-current assets as the Company does not intend to utilize them to fund current operations.

#### NOTE 6 - PROPERTY AND EQUIPMENT

Property and equipment consists of the following:

	June 30, 2017	December 31, 2016
	(in thousands)	
Leasehold improvements	\$19,898	\$12,131
Lab equipment	7,376	5,397
Office furniture	1,599	1,560
Manufacturing equipment	1,694	1,275
Computer and office equipment	947	623
Equipment held under capital leases	204	181
Software	136	85
Total	31,854	21,252
Less: accumulated depreciation	(6,396 )	(4,748 )
Property and equipment, net	\$25,458	\$16,504

During the six months ended June 30, 2017 and 2016, the Company recorded \$1.6 million and \$1.0 million of depreciation expense, respectively. Leasehold improvements as of June 30, 2017 includes \$2.5 million related to costs incurred by the landlord.

#### NOTE 7 – ACCRUED EXPENSES AND OTHER CURRENT LIABILITIES

Accrued expenses and other liabilities consist of the following:

	June 30, 2017	December 31, 2016
	(in thousands)	
Accrued construction costs	\$2,304	\$ 3,120
Accrued payroll	1,960	1,568
Accrued patient treatment costs	1,702	1,006
Accrued manufacturing costs	1,578	1,704
Accrued other	2,620	1,965
Total accrued expenses and other current liabilities	\$10,164	\$ 9,363



## NOTE 8 - 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 on the Closing Date. 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 the Company's assets, excluding intellectual property, but including proceeds from the sale, license, or disposition of our intellectual property. 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 9.60% and 10.10% at December 31, 2016 and June 30, 2017, respectively.

As a result of the additional borrowing on March 8, 2017, the interest only period was extended for an additional six months through March 2018. Beginning in April 2018, equal monthly payments of principal and interest are due over a 24 month period through the maturity date of March 1, 2020, upon which the remaining principal balance and the final facility charge of \$2.1 million will be due and payable.

The Company paid expenses related to the Loan Agreement of \$0.2 million, which, along with the final facility charge of \$2.1 million, have been recorded as deferred financing costs, which offset long-term debt on the Company's balance sheet. The deferred financing costs are being amortized over the term of the loan as interest expense. During the three and six months ended June 30, 2017, interest expense included \$222,000 and \$380,000, respectively, of amortized deferred financing costs. During the three and six months ended June 30, 2016, interest expense included \$122,000 and \$150,000, respectively, of amortized deferred financing costs.

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.

## NOTE 9 - STOCKHOLDERS' EQUITY

On March 29, 2017, the Company completed an underwritten public offering of 5,750,000 shares of its common stock at a price of \$12.00 per share, for an aggregate offering size of \$69.0 million, pursuant to a registration statement on Form S-3 (File No. 333-209012) that was declared effective by the SEC on February 1, 2016. The net proceeds to the Company, after deducting underwriting discounts, and commissions and offering expenses was approximately \$64.6 million. These costs have been recorded as a reduction of the proceeds received from the offering.

On June 28, 2017, the Company filed a Registration Statement on Form S-3 (File No. 333-219021) for the offer and sale by the Company of its securities in one or more offerings for up to an aggregate maximum offering price of \$150,000,000 (which includes \$81,000,000 of unsold securities that were previously registered under the Company's Registration Statement on Form S-3 (File No. 333-191819), which was filed on January 15, 2016 and declared effective on February 1, 2016). The SEC declared the registration statement effective on July 12, 2017.

## NOTE 10 - SHARE-BASED COMPENSATION PLANS

At June 30, 2017, the Company had share-based awards outstanding under four share-based compensation plans, as follows:

### 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. As of June 30, 2017, there were 146,210 shares of common stock reserved for issuance pursuant to outstanding options previously granted under the 2006 Plan. The 2006 Plan was terminated by the Board in October 2014.

### 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. As of June 30, 2017, there were 1,712,181 shares of common stock reserved for issuance pursuant to outstanding options previously granted under the 2011 Plan. The 2011 Plan terminated upon the effectiveness of the 2014 Plan described below.

### 2014 Equity Incentive Plan

The 2014 Equity Incentive Plan (the 2014 Plan) became effective in December 2014, upon the closing of the Company's initial public offering. 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.

On June 14, 2017, the stockholders approved an amendment to the 2014 Plan to, among other things, increase the number of shares of common stock authorized for issuance under the 2014 Plan by 3,100,000 shares and eliminate the prior provision in the 2014 Plan that allowed the Company's Board of Directors to reprice stock options without stockholder approval.

The aggregate number of shares of common stock that are authorized for issuance under the 2014 Plan is 6,090,354 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. As of June 30, 2017, there were 3,414,184 shares available for issuance under the 2014 Plan.

### 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 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. During the six months ended June 30, 2017 and 2016, 19,204 and 17,115 shares were purchased under the ESPP. As of June 30, 2017, there were 475,477 shares available for issuance under the ESPP.

A summary of activity within the ESPP follows:	Six months ended June 30, 2017	2016
	(amounts in thousands)	
Deductions from employees	\$157	\$188
Share-based compensation expense recognized	\$136	\$133
Remaining share-based compensation expense	\$357	\$134

### Share-Based Compensation Expense

The valuation of the share-based compensation awards is a significant accounting estimate that requires the use of judgments and assumptions that are likely to have a material impact on the financial statements. The fair value of option grants is determined using the Black-Scholes option-pricing model. Expected volatilities utilized in the model are based on implied volatilities from traded stocks of peer companies. Similarly, the dividend yield is based on historical experience and the estimate of future dividend yields. The risk-free interest rate is derived from the U.S. Treasury yield curve in effect at the time of grant. The expected term of the options is based on the average period the stock options are expected to remain outstanding. As the Company does not have sufficient historical information to develop reasonable expectations about future exercise patterns and post-vesting employment termination behavior, the expected term is calculated as the midpoint between the weighted-average vesting term and the contractual expiration period also known as the simplified method.

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

Six months  
ended June

	30,	
	2017	2016
Risk-free interest rate	2.09%	1.81%
Volatility	71.6%	72.0%
Expected life (years)	6.08	6.08
Expected dividend yield	— %	— %

At June 30, 2017, total compensation cost not yet recognized was \$24.9 million and the weighted-average period over which this amount is expected to be recognized is 2.3 years. During the three and six months ended June 30, 2017, the Company received cash proceeds from the exercise of stock options of approximately \$0.7 million and \$1.3 million, respectively. The aggregate intrinsic value of options exercised during the three and six months ended June 30, 2017 was \$0.6 million and \$2.1 million.



Share-based compensation expense by classification for the three and six months ended June 30, 2017 and 2016 are as follows:

	Three Months		Six Months	
	Ended June 30,		Ended June 30,	
	2017	2016	2017	2016
	(in thousands)		(in thousands)	
Research and development	\$ 1,484	\$ 1,374	\$ 3,068	\$ 2,760
General and administrative	1,706	1,744	3,486	3,423
Total	\$ 3,190	\$ 3,118	\$ 6,554	\$ 6,183

The following table summarizes the stock option and stock award activity for all stock plans during the six months ended June 30, 2017:

	Options and Inducement awards	Weighted-Average Exercise Price Per Share	Weighted-Average Contractual Life (in years)	(in thousands) Aggregate Intrinsic Value <sup>(1)</sup>
December 31, 2016 <sup>(2)</sup>	4,590,945	\$ 12.21	7.59	\$ 21,254
Granted <sup>(3)</sup>	1,020,850	\$ 11.35		
Exercised	(283,166 )	\$ 4.58		
Forfeited	(274,123 )	\$ 15.20		
Outstanding at June 30, 2017	5,054,506	\$ 12.31	7.57	\$ 15,305
Exercisable at June 30, 2017	2,621,427	\$ 10.41	6.59	\$ 12,424

<sup>(1)</sup> The aggregate intrinsic value is calculated as the fair value of restricted stock, restricted stock units and the difference between the exercise price of the underlying options and the estimated fair value of the common stock for the options that were in the money at June 30, 2017.

<sup>(2)</sup> At June 30, 2017 and December 31, 2016, there were 44,119 and 58,825 shares of unvested restricted common stock outstanding, respectively.

<sup>(3)</sup> Includes 500,000 of inducement option awards and 87,500 of restricted stock units granted in the during 2017.

#### NOTE 11 - COMMITMENTS AND CONTINGENCIES

##### Litigation

None.

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our Annual Report on Form 10-K for the year ended December 31, 2016, or our Annual Report, as well as our unaudited consolidated financial statements and related notes included in this Quarterly Report on Form 10-Q, or this Quarterly Report.

Forward-Looking Statements

This report contains forward-looking statements and information within the meaning of Section 27A of the Securities Act of 1933, as amended, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act, which are subject to the "safe harbor" created by those sections. These forward-looking statements include, but are not limited to, statements concerning our strategy, future operations, future financial position, future revenues, projected costs, prospects and plans and objectives of management. The words "anticipate," "believe," "could," "designed," "estimate," "expect," "intend," "may," "plan," "potential," "project," "will," "would," and similar expressions are used to identify forward-looking statements, although not all forward-looking statements contain these identifying words. We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements that we make. These forward-looking statements involve risks and uncertainties that could cause our actual results to differ materially from those in the forward-looking statements, including, without limitation, the risks set forth in Part II, Item 1A, "Risk Factors" in the Quarterly Report on Form 10-Q, filed May 8, 2017, Part I, Item 1A, "Risk Factors" in our Annual Report and in our other filings with the SEC. The forward-looking statements are applicable only as of the date on which they are made, and we do not assume any obligation to update any forward-looking statements.

Overview

We are a clinical stage biopharmaceutical company focused on discovering and developing novel cellular immunotherapies for various forms of cancer, including both hematological cancers and solid tumors, as well as orphan inherited blood disorders. We are using our proprietary Chemical Induction of Dimerization, or CID technology platform to engineer 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, enhanced CAR T cell approaches are being developed 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 candidates, 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 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, with events defined as transplant-related or non-relapse mortality (severe GvHD and serious 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 are finalizing plans for future U.S. clinical trials of BPX-501. We plan to pursue one or more clinical trials with the intent of filing for FDA approval, partially supported by a \$16.9 million award from the Cancer Prevention and Research Institute of Texas, or CPRIT.

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.

**CD19 CAR-T Program** - We are working with academic collaborators to establish clinical proof of concept for CaspaCIDE® in the CD19 setting. We believe that this strategy allows a cost-effective approach for clinical evaluation of differentiated product candidates in the highly competitive landscape of CD19-targeted therapies in development. As part of this strategy, in November 2016 we announced an expanded collaboration with Ospedale Pediatrico Bambino Gesù, a leading European pediatric research center and hospital, and expects a CaspaCIDE-enabled CD19 CAR-T cell therapy to enter clinical development in the second half of 2017.

We have developed an efficient and scalable process to manufacture genetically modified T cells of high quality, which T cells are currently being manufactured in-house and 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.

#### Critical Accounting Policies and Estimates

The preparation of consolidated financial statements in conformity with generally accepted accounting principles requires us to make judgments, estimates and assumptions in the preparation of our consolidated financial statements and accompanying notes. Actual results could differ from those estimates. We believe there have been no material changes in our critical accounting policies as discussed in our Annual Report.

## Financial Operations Overview

### Grant Revenue

To date, we have only recognized revenue from government grants and we have not generated any product revenue. Grant funds are received 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 3 to the unaudited condensed consolidated financial statements contained herein.

### 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 \$1.3 million has been received. We accrued the revenue based on the costs incurred for the programs associated with the grant. The fourth year of the grant expired March 31, 2017.

### 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 in Note 3 to the unaudited condensed consolidated financial statements included herein.

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. Thus, 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;
- the efficacy and safety profile of the product candidates; and
- the ability to successfully manufacture patient doses

In addition, the potential for success of each product candidate will depend on numerous factors, including clinical trial outcomes, competition, manufacturing capability and commercial viability. We determine which programs to pursue and how much to fund each program in response to ongoing scientific assessments, competitive developments, clinical trial results, 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.

#### 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.

As our research and development activities continue to expand, we also expect general and administrative expenses to increase due to the anticipated need for additional supporting infrastructure, primarily personnel costs. We also expect general and administrative expenses to increase as we prepare for commercialization of BPX-501, continue to evaluate the commercial opportunities for BPX-601 and BPX 701, and as we begin operations in our European subsidiary.

#### Income Taxes

We did not recognize any income tax expense for the three and six months ended June 30, 2017.



## Results of Operations

## Comparison of the Three and Six Months Ended June 30, 2017 and 2016

The following table sets forth our results of operations for the three and six months ended June 30, 2017 and 2016:

	Three Months Ended June 30,			Six Months Ended June 30,		
	2017	2016	Change	2017	2016	Change
	(in thousands)			(in thousands)		
Grant revenues	\$—	\$101	\$(101)	\$128	\$193	\$(65)
Operating expenses:						
Research and development	17,959	12,031	5,928	33,254	22,889	10,365
License fees	343	150	193	698	280	418
General and administrative	5,486	4,179	1,307	11,413	8,463	2,950
Total operating expenses	23,788	16,360	7,428	45,365	31,632	13,733
Loss from operations	(23,788)	(16,259)	(7,529)	(45,237)	(31,439)	(13,798)
Other income (expense):						
Interest income	307	236	71	504	463	41
Interest expense	(976)	(486)	(490)	(1,697)	(608)	(1,089)
Total other income (expense)	(669)	(250)	(419)	(1,193)	(145)	(1,048)
Net loss	\$(24,457)	\$(16,509)	\$(7,948)	\$(46,430)	\$(31,584)	\$(14,846)

## Research and Development Expenses

Research and development expenses were \$18.0 million and \$12.0 million for the three months ended June 30, 2017 and June 30, 2016, respectively. The \$5.9 million increase in research and development expenses for the three months ended June 30, 2017, was due to an increase in clinical and manufacturing costs of \$2.5 million related to BPX-501, primarily due to increased patient enrollment in our clinical trials. Increased research and development expenses in the three months ended June 30, 2017 also includes \$0.3 million under collaboration agreements, and \$3.3 million in increased personnel, overhead charges and manufacturing start-up expenses. Expenses related to BPX-601 were approximately \$0.7 million higher in the 2016 period, as we were performing pre-clinical, process development and regulatory work in preparation for beginning clinical trials.

Research and development expenses were \$33.3 million and \$22.9 million for the six months ended June 30, 2017 and 2016, respectively. The \$10.4 million increase in research and development expenses for the six months ended June 30, 2017, included an increase in clinical and manufacturing costs of \$5.4 million related to BPX-501, primarily due to increased patient enrollment in our clinical trials. The increase in research and development expenses is also due to increased expenses of approximately \$0.8 million under collaboration agreements, (see Note 12 to the financial statements included in our Annual Report), and \$4.8 million for increased personnel, overhead charges and manufacturing facility start-up costs. Expenses related to BPX-601 were approximately \$0.8 million higher in the 2016 period, as we were performing pre-clinical, process development and regulatory work in preparation for beginning clinical trials.

The following table presents our research and development expense by project/category for the three and six months ended June 30, 2017 and 2016:

	Three Months Ended June 30,			Six Months Ended June 30,		
	2017	2016	Change	2017	2016	Change
	(in thousands)			(in thousands)		
Product Candidates						
BPX-501	\$9,754	\$7,297	\$2,457	\$17,746	\$12,355	\$5,391
BPX-601	596	1,310	(714)	1,117	1,899	(782)
BPX-701	871	68	803	1,158	280	878
General	6,738	3,356	3,382	13,233	8,355	4,878

Total \$17,959 \$12,031 \$5,928 \$33,254 \$22,889 \$10,365

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#### License Fees

We incurred license fees of \$0.3 million and \$0.2 million in the three months periods ended June 30, 2017 and 2016, respectively, under the terms of our various license agreements for intellectual property. In the six month periods ended June 30, 2017 and 2016, license fees incurred were \$0.7 million and \$0.3 million, respectively. See "Contractual Obligations and Commitments" below and Note 12 to the audited financial statements in our Annual Report for additional information about our license agreements.

#### General and Administrative Expenses

General and administrative, or G&A, expenses were \$5.5 million and \$11.4 million for the three and six months ended June 30, 2017, respectively, and \$4.2 million and \$8.5 million for the three and six months ended June 30, 2016, respectively. The increase in G&A expenses of \$1.3 million and \$3.0 million for the three and six months ended June 30, 2017, respectively, was primarily due to our overall growth, including an increase in personnel related costs, primarily due to hiring additional employees and severance costs, higher facility costs and increased legal, accounting and travel expenses. We believe our future general and administrative expenses will continue to increase as the Company continues to grow and expand its operations.

#### 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 June 30, 2017 and December 31, 2016, we had cash, cash equivalents, restricted cash and investment securities of \$139.0 million and \$113.4 million, respectively. Cash in excess of immediate requirements is invested in accordance with our investment policy, primarily with a view to liquidity and capital preservation.

On June 28, 2017, the Company filed a registration statement on Form S-3 (File No. 333-219021) for the offer and sale by the Company of its securities in one or more offerings for up to an aggregate maximum offering price of \$150,000,000 (which includes \$81,000,000 of unsold securities that were previously registered under the Company's registration statement on Form S-3 (File No. 333-191819), which was filed on January 15, 2016 and declared effective on February 1, 2016). The SEC declared the registration statement effective on July 12, 2017.

On March 29, 2017, we completed an underwritten public offering of 5,750,000 shares of our common stock at a price of \$12.00 per share, for an aggregate offering size of \$69.0 million, pursuant to a registration statement on Form S-3 (File No. 333-209012) that was declared effective by the SEC on February 1, 2016. The net proceeds to us, after deducting underwriting discounts and commissions and offering expenses, was approximately \$64.6 million.

On March 10, 2016, we entered into a term loan arrangement with Hercules Capital, 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. As a result of the additional borrowing on March 8, 2017, the interest only period was extended for an additional six months 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%. The interest rate on the loan was 9.35% and 10.1% at June 30, 2016 and 2017, respectively. For additional information about the loan, see Note 8 to the unaudited consolidated condensed financial statements included herein.

## Cash Flows

The following table sets forth a summary of our cash flows for the six months ended June 30, 2017 and 2016:

	Six Months Ended June 30,		
	2017	2016	Change
	(in thousands)		
Net cash used in operating activities	\$(42,623)	\$(24,550)	\$(18,073)
Net cash provided by (used in) investing activities	3,427	(11,409)	14,836
Net cash provided by financing activities	75,947	15,267	60,680
Net change in cash, cash equivalents, and restricted cash	\$36,751	\$(20,692)	\$57,443

## Operating Activities

Net cash used in operating activities for the six months ended June 30, 2017 was comprised of a net loss of \$46.4 million, which included share-based compensation expense of \$6.6 million, and depreciation expense of \$1.6 million and amortization of deferred financing costs of \$0.4 million. Net cash used in operating activities also included an increase in prepaid expenses and other assets of \$1.2 million, a decrease in accounts payable and other liabilities of \$3.7 million, primarily due to completion of the first phase of construction of our manufacturing facility.

Net cash used in operating activities for the six months ended June 30, 2016 was comprised of a net loss of \$31.6 million, which included share-based compensation expense of \$6.2 million and depreciation expense of \$1.0 million and amortization of deferred financing costs of \$0.2 million. Net cash used in operating activities was also primarily comprised of the following primary components: a decrease in receivables of \$0.1 million, a decrease in prepaid expenses and other assets of \$0.3 million and a decrease in accounts payable and other liabilities of \$0.8 million.

## Investing Activities

Net cash provided by investing activities for the six months ended June 30, 2017 was \$3.4 million, consisting of the proceeds from sale of investment securities of \$39.0 million, offset by the purchase of investment securities of \$28.0 million and the purchase of property and equipment of \$7.6 million.

Net cash used in investing activities for the six months ended June 30, 2016 was \$11.4 million, consisting of the purchase of investment securities of \$22.7 million, offset by the proceeds from sale of investment securities of \$15.9 million and the purchase of property and equipment of \$4.6 million.

## Financing Activities

Net cash provided by financing activities for the six months ended June 30, 2017 was \$75.9 million, which was derived from \$64.6 million in net proceeds from our public offering in the first quarter, borrowings on long-term debt of \$10.0 million, proceeds from the exercise of stock options of \$1.3 million, proceeds from the issuance of stock under the employee stock purchase plan of \$0.2 million, reduced by the payment of debt issuance costs of \$0.1 million.

Net cash provided by financing activities for the six months ended June 30, 2016 was \$15.3 million, which was derived primarily from borrowings of long-term debt of \$15.0 million and proceeds from the issuance of stock under the employee stock purchase plan of \$0.2 million.

## 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;
- market acceptance of our products, if and when approved; and
- successfully negotiating reimbursement for our products from various third-party payors;
- the ability to successfully manufacture patient doses

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.

## 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 June 30, 2017 will enable us to fund our operating expenses and capital expenditure requirements through 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; and
- 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.

## Contractual Obligations and Commitments

Our contractual obligations as of June 30, 2017 were as follows:

	(in thousands)				
	Commitments Less than 1 Year	1 to 3 Years	3 to 5 Years	More Than 5 Years	
License agreements (1)	\$67,147	\$793	\$8,279	\$16,564	\$41,511
Long-term debt obligations (2)	32,085	3,412	28,673	—	—
Operating lease agreements (3)	12,732	2,007	3,706	2,111	4,908
Manufacturing build-out obligation (4)	3,412	3,412	—	—	—
Research collaborations (5)	1,093	729	364	—	—
Manufacturing arrangements (6)	6,967	4,913	685	684	685
Sponsored research agreements (7)	2,624	1,369	1,255	—	—
Equipment capital lease agreements (8)	280	68	135	77	—
Total contractual obligations	\$126,340	\$16,703	\$43,097	\$19,436	\$47,104

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.

(2) Long-term debt obligations - Obligations under our debt facility. See Note 8 to the unaudited condensed consolidated financial statements included herein.

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Operating lease agreements - The amounts above are comprised of one five-year lease agreement and one 11-year (3) 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 in our Annual Report.

(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.

(5) Research collaborations - We entered into a research collaboration with Ospedale Pediatrico Bambino Gesù (OPBG), a leading European pediatric research center and hospital, with commitments through 2018.

(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 three 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 several office equipment lease agreements with various (8) terms. The commitments include equipment, maintenance and supplies. See Note 12 to the financial statements included in our Annual Report.

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.

#### Recent Accounting Pronouncements

See Note 3 to the Notes to Unaudited Condensed Consolidated Financial Statements in “Item 1 - Financial Statements” in this Quarterly Report for discussion regarding recent accounting pronouncements.

#### Off-Balance Sheet Arrangements

During the periods presented, we did not have, and we do not currently have, any off-balance sheet arrangements, as defined in the rules and regulations of the Securities and Exchange Commission.

#### Item 3. Quantitative and Qualitative Disclosures about Market Risks

The primary objective of our investment activities is to preserve our capital and meet our liquidity needs to fund operations. We also seek to generate competitive rates of return from our investments without assuming significant risk. To achieve our objectives, we maintain a portfolio of cash equivalents and investments in a variety of securities that are of high credit quality based on ratings from commonly relied upon rating agencies. As of June 30, 2017, we had cash, cash equivalents, restricted cash and investment securities of \$139.0 million. Our cash equivalents and investments in investment securities may be subject to interest rate risk and could fall in value if market interest rates increase. However, because our cash is invested in accounts with market interest rates and because our cash equivalents and investments in investment securities are traded in active markets, we believe that our exposure to interest rate risk is not significant and estimate that an immediate and uniform 10% increase in market interest rates from levels as of June 30, 2017 would not have a material impact on the total fair value of our portfolio.

We sometimes contract for the conduct of clinical trials or other research and development and manufacturing activities with contract research organizations, clinical trial sites and contract manufacturers in Europe, and in the future potentially elsewhere outside of the United States. We may be subject to exposure to fluctuations in foreign



currency exchange rates in connection with these agreements. If the average exchange rate between the currency of our payment obligations under any of these agreements and the U.S. dollar were to strengthen or weaken by 10% against the corresponding exchange rate as of June 30, 2017, we estimate that the impact on our financial position, results of operations and cash flows would not be material. We do not hedge our foreign currency exposures.

We have not used derivative financial instruments for speculation or trading purposes.

#### Item 4. Controls and Procedures

##### Management's Evaluation of our 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 defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, as of June 30, 2017. 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 June 30, 2017, our Chief Executive Officer and Chief Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective.

##### 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.

## PART II. OTHER INFORMATION

### Item 1. Legal Proceedings

None.

### Item 1A. Risk Factors

Our business and results of operations are subject to a number of risks and uncertainties. You should carefully consider the risk factors described under the heading “Risk Factors” in our Annual Report and in other reports we file with the SEC. The occurrence of any of the risks described in our Annual Report or herein 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 Quarterly Report and those we may make from time to time. You should consider all of the risk factors described in our Annual Report and our Quarterly Report on Form 10-Q, filed May 8, 2017, when evaluating our business. Other than risk factors included in Item 1A of our Quarterly Report on Form 10-Q filed on May 8, 2017, there have been no material changes to the risk factors included in Item 1A of our Annual Report.

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

#### Recent Sales of Unregistered Securities

None.

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

On December 23, 2014, we completed the initial public offering of our common stock pursuant to a registration statement on Form S-1 (File Nos. 333-200328 and 333-201031), which was declared effective by the SEC on December 17, 2014. Since the effective date of our registration statement through June 30, 2017, we have used approximately \$149.0 million of the net offering proceeds to fund our operating activities, and the remainder is invested in cash and cash equivalent securities, or highly-liquid investment securities.

#### Purchase of Equity Securities

We did not purchase any of our registered securities during the period covered by this Quarterly Report.

### Item 5. Other Information

(a)

On August 3, 2017, we entered into a letter agreement with Alan A. Musso, our Chief Financial Officer and Treasurer, pursuant to which we agreed to pay Mr. Musso a one-time lump sum cash bonus of \$192,500, less required withholdings. Mr. Musso’s eligibility to receive the cash bonus is conditioned upon Mr. Musso remaining in our continuous employment through April 30, 2018 and Mr. Musso not giving us notice prior to April 30, 2018 of his intent to resign from his employment with us, provided that Mr. Musso shall remain eligible to receive the bonus if we terminate his employment prior to April 30, 2018 for reasons other than for cause (as defined in his employment agreement dated December 4, 2014). The bonus is payable to Mr. Musso on the first regularly scheduled payroll date following the earlier of April 30, 2018 or, if terminated without cause, the effective date of the release signed by Mr. Musso as required by his employment agreement.

Item 6. Exhibits

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

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Signatures

Pursuant to the requirements 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: August 8, 2017 By: /s/ Richard A. Fair  
Richard A. Fair  
President and Chief Executive Officer

Date: August 8, 2017 By: /s/ Alan A. Musso  
Alan A. Musso  
Chief Financial Officer and Treasurer  
Principal Financial and Accounting Officer

EXHIBIT INDEX

Exhibit number	Description of exhibit
3.1(1)	Amended and Restated Certificate of Incorporation of the Registrant.
3.2(1)	Amended and Restated Bylaws of the Registrant.
4.1	Reference is made to Exhibits 3.1 and 3.2.
4.2(2)	Form of Common Stock Certificate of the Registrant.
4.3(2)	Second Amended and Restated Investor Rights Agreement by and among the Registrant and certain of its stockholders, dated August 22, 2014.
4.4(3)	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	Separation and Consulting Agreement, by and between the registrant and Annemarie Moseley, Ph.D., M.D., effective May 10, 2017.
10.2	Amended and Restated Employment Agreement, by and between the registrant and Alan J. Smith, Ph.D., effective May 10, 2017.
31.1	Certification of Chief Executive Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2	Certification of Chief Financial Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1	Certifications of Chief Executive Officer and Chief Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	XBRL Taxonomy Extension Label Linkbase Document
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document
(1)	Incorporated by reference to the Registrant's Current Report on Form 8-K, filed with the SEC on December 23, 2014.
(2)	Incorporated by reference to the Registrant's Registration Statement on Form S-1 (File No. 333-200328), as amended, originally filed with the SEC on November 18, 2014.

- (3) Incorporated by reference to the Registrant's Annual Report on Form 10-K, filed with the SEC on March 14, 2016.

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