# UNITED STATES SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

# **FORM 10-Q**

(Mark One)

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

For the quarterly period ended September 30, 2015

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

(State or other jurisdiction of incorporation or organization)

2836

(Primary Standard Industrial Classification Code Number) **20-1450200** (I.R.S. Employer 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** x **No** o

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** x **No** o

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

Large accelerated filer	0		Accelerated filer	0
Non-accelerated filer	х	(Do not check if a smaller reporting company)	Smaller reporting company	0

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). **Yes** o **No** x As of October 30, 2015, there were 26,501,110 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.

# **Balance Sheets**

# (In thousands, except share and par value amounts)

September 30, 2 (Unaudited)			Dece	ember 31, 2014
ASSETS				
Current assets:				
Cash and cash equivalents	\$	92,487	\$	191,602
Investment securities, available for sale - short-term		20,744		—
Accounts receivable, interest and other receivables		388		298
Prepaid expenses and other current assets		1,702		1,322
Total current assets		115,321		193,222
Investment securities, available for sale - long-term		50,018		—
Property and equipment, net of accumulated depreciation		6,323		2,427
Other assets		446		145
TOTAL ASSETS	\$	172,108	\$	195,794
LIABILITIES AND STOCKHOLDERS' EQUITY				
Current liabilities:				
Accounts payable	\$	1,534	\$	1,209
Accrued expenses and other liabilities		3,315		2,163
Deferred revenue				13
Current portion of deferred rent		170		97
Current portion of deferred manufacturing costs		_		154
Total current liabilities		5,019		3,636
Long-term liabilities:				
Deferred rent and other liabilities		984		209
Deferred manufacturing costs		_		313
Total long-term liabilities		984		522
TOTAL LIABILITIES		6,003		4,158
Commitments and contingencies: (Note: 10)				
Stockholders' equity:				
Common stock, \$0.01 par value; 200,000,000 shares authorized at September 30, 2015 and December 31, 2014, respectively; 27,178,328 shares issued and 26,500,865 shares issued and outstanding at September 30, 2015; 27,050,055 issued and 26,372,592 issued and outstanding at December 31, 2014		272		271
Treasury stock: 677,463 shares held at September 30, 2015 and December 31, 2014		(5,056)		(5,056)
Additional paid-in capital		315,736		309,365
Accumulated other comprehensive loss		(204)		
Accumulated deficit		(144,643)		(112,944)
Total stockholders' equity		166,105		191,636
Total liabilities and stockholders' equity	\$	172,108	\$	195,794
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See accompanying notes, which are an integral part of these unaudited financial statements.

# Bellicum Pharmaceuticals, Inc.

# **Statements of Operations and Comprehensive Loss**

# (In thousands, except share and per share amounts)

# (Unaudited)

		Three months ended September 30,				ine months end	ed September 30,	
		2015 2014			2015			2014
REVENUES								
Grants	\$	57	\$	660	\$	248	\$	1,766
Total revenues		57		660		248		1,766
OPERATING EXPENSES								
Research and development (includes share-based compensation of \$966 and \$7 for the three months ended September 30, 2015 and 2014, respectively an \$2,526 and \$216 for the nine months ended September 30, 2015 and 2014 respectively)	d	9,792		2,257		23,522		7,881
General and administrative (includes share-based compensation of \$1,336 and \$1 for the three months ended September 30, 2015 and 2014, respectively and \$3,412 and \$30 for the nine months ended September 30, 2015 and 2014, respectively)	1	3,882		1,300		8,856		2,332
Total operating expenses		13,674	-	3,557		32,378		10,213
Loss from operations		(13,617)		(2,897)		(32,130)		(8,447)
OTHER INCOME (EXPENSE):								
Interest income		211		9		432		15
Interest expense				(11)		—		(38)
Change in fair value of warrant liability		—		(1,197)		—		(1,197)
Other		(2)		_		(2)	_	—
Total other income (expense)		209		(1,199)		430		(1,220)
NET LOSS	\$	(13,408)	\$	(4,096)	\$	(31,700)	\$	(9,667)
Preferred stock dividends		_		(328)				(1,432)
Net loss attributable to common shareholders, basic and diluted	\$	(13,408)	\$	(4,424)	\$	(31,700)	\$	(11,099)
Net loss per common share attributable to common shareholders, basic and diluted	\$	(0.51)	\$	(2.08)	\$	(1.21)	\$	(5.45)
Weighted-average shares outstanding, basic and diluted		26,376,456		2,124,247	\$ 2	26,301,914	\$	2,036,691
					_			
Net loss	\$	(13,408)	\$	(4,096)	\$	(31,700)	\$	(9,667)
Other comprehensive loss:								
Unrealized loss on investment securities						(204)		
Comprehensive loss	\$	(13,408)	\$	(4,096)	\$	(31,904)	\$	(9,667)

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

# Bellicum Pharmaceuticals, Inc.

# **Statements of Cash Flows**

### (In thousands)

# (Unaudited)

	Nine months ended September 30,			
	 2015		2014	
CASH FLOWS FROM OPERATING ACTIVITIES				
Net loss	\$ (31,700)	\$	(9,667)	
Adjustments to reconcile net loss to net cash used in operating activities:				
Depreciation expense	769		485	
Share-based compensation	5,938		246	
Amortization of lease liability	(54)		(72)	
Amortization of premium on investment securities, net	377		—	
Loss on warrant liability	—		1,197	
Loss on disposition of investment securities	14		—	
Loss on disposition of fixed assets	2			
Changes in operating assets and liabilities:				
Accounts receivable	(90)		(475)	
Prepaid expenses and other assets	(681)		232	
Accounts payable	325		1,142	
Accrued and other liabilities	1,131		(490)	
Deferred costs	379		244	
NET CASH USED IN OPERATING ACTIVITIES	(23,590)		(7,158)	
CASH FLOWS FROM INVESTING ACTIVITIES				
Purchase of investment securities	(79,100)			
Proceeds from sale of investment securities	7,743		_	
Purchases of property and equipment	(4,597)		(401)	
CASH USED IN INVESTING ACTIVITIES	(75,954)		(401)	
CASH FLOWS FROM FINANCING ACTIVITIES				
Proceeds from issuance of common stock	442		212	
Proceeds from issuance of preferred stock	_		62,320	
Payment of issuance costs	(8)		(3,524)	
Payment on capital lease obligation	(5)			
Payment of deferred offering costs	_		(466)	
Proceeds from line of credit	_		82	
Payments on line of credit	_		(300)	
NET CASH PROVIDED BY FINANCING ACTIVITIES	429		58,324	
NET CHANGE IN CASH AND CASH EQUIVALENTS	 (99,115)		50,765	
CASH AND CASH EQUIVALENTS AT BEGINNING OF PERIOD	191,602		11,167	
CASH AND CASH EQUIVALENTS AT END OF PERIOD	\$ 92,487	\$	61,932	
NON-CASH INVESTING AND FINANCING ACTIVITIES				
Dividends accreted on preferred stock	\$ _	\$	1,432	
Capital lease obligation	\$ 65	\$	_	

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

#### **Bellicum Pharmaceuticals, Inc.**

#### Notes to Unaudited Financial Statements

#### NOTE 1 - ORGANIZATION AND BUSINESS DESCRIPTION

Bellicum Pharmaceuticals, Inc. (the Company or Bellicum), was incorporated in Delaware in July 2004 and is based in Houston, Texas. The Company is a clinical stage biopharmaceutical company focused on discovering and developing novel cellular immunotherapies for various forms of cancer, including both hematological cancers and solid tumors, as well as orphan inherited blood disorders. The Company is devoting substantially all of its present efforts to developing next-generation product candidates in some of the most important areas of cellular immunotherapy, including, hematopoietic stem cell transplantation, CAR-T and TCR cell therapy. The Company has not generated any revenue from product sales to date and does not anticipate generating revenues from product sales in the foreseeable future.

The Company is subject to risks common to companies in the biotechnology industry and the future success of the Company is dependent on its ability to successfully complete the development of, and obtain regulatory approval for, its product candidates, manage the growth of the organization, obtain additional financing necessary in order to develop launch and commercialize its product candidates, and compete successfully with other companies in its industry.

#### **NOTE 2 - SIGNIFICANT ACCOUNTING POLICIES**

#### **Basis of Presentation**

The accompanying interim financial statements are unaudited. These unaudited interim financial statements have been prepared in accordance with U.S. generally accepted accounting principles (GAAP) and following 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 financial statements have been prepared on the same basis as the audited financial statements and include all adjustments, which include only normal recurring 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. These statements do not include all disclosures required by GAAP and should be read in conjunction with the Company's Annual Report on Form 10-K filed for the fiscal year ended December 31, 2014 (the Annual Report). A copy of the Annual Report is available on the SEC's website, www.sec.gov, under the Company's ticker symbol (BLCM) or on Bellicum's website, 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).

# Use of Estimates

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

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

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

#### Reclassifications

Certain research and development indirect costs, including facilities and overhead, were previously included in general and administrative costs. These research and development indirect costs are included in research and development expense in the three and nine months ended September 30, 2015, and results for the three and nine months ended September 30, 2014 have been reclassified to conform to the current year presentation. The effect of the reclassification of the results for the three and nine months ended September 30, 2014 was to increase research and development expense and reduce general and administrative expense by \$0.1 million and \$0.8 million, respectively, with no change in total operating expense or net loss.

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

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

	As of Septer	nber 30,
Common Stock Equivalents:	2015	2014
Series A Convertible preferred stock - as converted to common stock		1,496,782
Series B Convertible preferred stock - as converted to common stock	—	4,791,740
Series C Convertible preferred stock - as converted to common stock	—	5,936,297
Warrants to purchase Series C Convertible preferred stock - as converted to common stock	—	3,858,549
Warrants to purchase common stock	355,392	473,031
Unvested shares of restricted stock	117,647	—
Options to purchase common stock	3,547,949	1,602,339
	4,020,988	18,158,738

#### **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 and 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 at the time of purchase and reevaluates its classification as of each balance sheet date. All investment securities owned during the nine months ended September 30, 2015, were 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 included in stockholders' equity. Interest and dividend income on investment securities, accretion of discounts and amortization of premiums and realized gains and losses are included in interest income in the Statements of Operations and Comprehensive Income Loss.

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

#### NOTE 3 - 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 OSC 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 September 30, 2015 and December 31, 2014, respectively (in thousands):

		Fair Value Measurements at Reporting Date Using					
	Balance at September 30, 2015		Quoted prices in active markets for identical assets (Level 1)		ignificant other oservable inputs (Level 2)		Significant unobservable ıputs (Level 3)
Cash Equivalents:							
Money market funds	\$ 65,211	\$	65,211	\$		\$	_
Government sponsored securities	16,059		—		16,059		
Commercial paper	2,625		—		2,625		_
Total Cash Equivalents	\$ 83,895	\$	65,211	\$	18,684	\$	—
Investment Securities:							
U.S. Treasury and state government agency-backed securities	\$ 13,834	\$	—	\$	13,834	\$	
Corporate debt securities	50,701		—		50,701		_
Municipal bonds	6,227		—		6,227		
Total Investment Securities	\$ 70,762	\$		\$	70,762	\$	_

			Fair Value Measurements at Reporting Date Using						
	Balance at December 31, 20	14	markets for identical observa			gnificant other servable inputs (Level 2)	uno	gnificant bservable ts (Level 3)	
Cash Equivalents:									
Money market funds	43,5	87		43,587	\$		\$	_	
Total Cash Equivalents	\$ 43,5	87	\$	43,587	\$	_	\$	_	

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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 September 30, 2015 (in thousands):

	September 30, 2015									
Investment Securities:		ortized Cost	G	Fross Unrealized Gains	Gro	oss Unrealized Losses		egate Estimated Fair Value		
U.S. Treasury and U.S. or state government agency-backed securities	\$	13,836	\$	2	\$	(4)	\$	13,834		
Corporate debt securities		50,899		19		(217)		50,701		
Municipal bonds		6,231		1		(5)		6,227		
Total Investment Securities	\$	70,966	\$	22	\$	(226)	\$	70,762		

The Company's investment securities as of September 30, 2015, will reach maturity between October 2015 and July 2026, with a weighted-average maturity date in November 2016. There were no investment securities at December 31, 2014.

#### NOTE 4 – ACCRUED EXPENSES

Accrued liabilities consist of the following (in thousands):

	September 30, 2015			December 31, 2014
Accrued payroll	\$	721	\$	731
Commission on exercise of warrants		—		731
Medical facility fees		1,627		201
Patient treatment costs		342		128
Other		625		372
Total accrued expenses	\$	3,315	\$	2,163

## **NOTE 5 - DEFERRED MANUFACTURING COSTS**

At December 31, 2014, the Company had deferred manufacturing costs of \$0.3 million and a credit against future manufacturing costs of \$0.2 million, pursuant to a service agreement with a third party manufacturer. The deferred manufacturing cost was amortized as manufacturing batches of cell therapy products were produced and the credit was recognized monthly over the term of the agreement. In September 2015, the Company and the third party manufacturer agreed to amend the service agreement, pursuant to which both parties were released from all prior obligations except for a 30-day transition period during which the third party would continue to manufacture batches in process, not to exceed three batches. As a result of the amendment to the agreement, and the release of its obligations, the Company reversed the remaining deferred manufacturing costs and credit against future manufacturing costs, resulting in a decrease of \$0.4 million in research and development expenses in the three and nine month periods ended September 30, 2015. See Note 8 to the audited financial statements included in the Annual Report.

### **NOTE 6 - STOCKHOLDERS' EQUITY**

#### **Preferred Stock**

As of September 30, 2015 and December 31, 2014, the Company had 10,000,000 authorized shares of preferred stock, with none outstanding and a par value of \$0.01 per share.

#### **Common Stock**

As of September 30, 2015 and December 31, 2014, the Company had 200,000,000 authorized shares of common stock with a par value of \$0.01 per share.

#### **Reverse Stock Split**

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

#### **NOTE 7 - SHARE-BASED COMPENSATION**

At September 30, 2015, the Company had share-based awards outstanding under four share-based compensation plans as follows:

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 September 30, 2015, 158,292 shares of common stock were reserved for issuance pursuant to outstanding options previously granted under the 2006 Plan to purchase common stock of the Company. The 2006 Plan was terminated by the Board in October 2014.

The 2011 Stock Option Plan (the 2011 Plan) provided for the issuance of incentive and non-qualified stock options to employees, including officers, nonemployee directors and consultants to the Company. As of September 30, 2015, 2,315,881 shares of common stock were reserved for issuance pursuant to outstanding options previously granted under the 2011 Plan to purchase common stock of the Company. The 2011 Plan terminated upon the effectiveness of the 2014 Plan described below.

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. The aggregate number of shares of common stock that are authorized for issuance under the 2014 Plan is 2,990,354 shares, plus any shares subject to outstanding options that were granted under the 2011 Plan or 2006 Plan that are forfeited, terminated, expired or are otherwise not issued.

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 our 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 our common stock, pursuant to purchase rights granted to our employees. The ESPP was approved by our board of directors and our stockholders in December 2014 and employee payroll deductions of approximately \$101,000 and \$296,000, respectively, were withheld during the three and nine months ended September 30, 2015. During the nine months ended September 30, 2015, 9,829 shares were purchased pursuant to the ESPP and the Company received \$159,000 in proceeds. There were no ESPP stock purchases during the three months ended September 30, 2015. The Company recorded share-based compensation expense for shares purchased for less than fair market value under the ESPP of \$58,000 and \$166,000, during the three and nine months ended September 30, 2015, respectively. There was \$0.3 million of unrecognized compensation expense related to the ESPP as of September 30, 2015, which will be recognized over the remaining 15 months of the plan.

The Company granted options to purchase 86,500 and 933,600 shares of its common stock during the three and nine months ended September 30, 2015, respectively. The fair value of the option grants during the three and nine months ended September 30, 2015 and 2014 was estimated at the date of grant using the Black-Scholes option pricing model with the following weighted-average assumptions:

	Nine months ended S	eptember 30,
	2015	2014
Expected volatility	87.3%	100.6%
Expected term (in years)	6.08	6.25
Risk-free interest rate	1.70%	2.61%
Expected dividend yield	%	%

At September 30, 2015, there was \$27.9 million of unrecognized compensation expense related to unvested stock options and stock that is expected to be recognized over a weighted-average period of 3.2 years.

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During the three and nine months ended September 30, 2015, the Company received cash proceeds from the exercise of stock options of approximately \$200,000 and \$283,000, respectively. The aggregate intrinsic value of options exercised during the three and nine months ended September 30, 2015 was \$1.5 million and \$2.3 million, respectively.

The following table summarizes the stock option activity for all stock plans during the nine months ended September 30, 2015:

	Options	Weighted- Average Exercise Price Per Share		Average Weighted- Exercise Average Price Contractual		(in thousands) Aggregate Intrinsic Value (1)
Outstanding at December 31, 2014	2,733,793	\$	5.09	8.39	\$	49,076
Granted	933,600	\$	23.09			
Exercised	(118,444)	\$	2.39			
Canceled or forfeited	(1,000)	\$	23.36			
Outstanding at September 30, 2015	3,547,949	\$	9.91	8.18	\$	25,005
Exercisable at September 30, 2015	1,388,121	\$	2.48	6.58	\$	16,805

<sup>(1)</sup> The aggregate intrinsic value is calculated as 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 September 30, 2015.

At September 30, 2015 and December 31, 2014, there were 117,647 shares of unvested common stock outstanding.

#### **NOTE 8 - GRANT REVENUE**

#### **CPRIT** Grant

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

During the three and nine months ended September 30, 2014, the Company incurred \$0.6 million and \$1.5 million, respectively, of expenses under the Grant Contract. As of September 30, 2015 and December 31, 2014, the Company had an outstanding grant receivable of \$0 and \$0.3 million respectively, for grant expenditures that were paid but had not yet been reimbursed.

#### NIH Grant

During each of the years 2015 and 2014, the Company was awarded \$0.3 million under a grant from the National Institutes of Health (NIH). The awards cover the period from April 2014 through March 2016. The awards were made pursuant to the authority of 42 USC 241 42 CFR 52, and are subject to the requirements of the statute. Funds spent on the grant are reimbursed through monthly reimbursement requests. During the three and nine months ended September 30, 2015, funds spent under the grant were \$0.1 million and \$0.2 million, respectively, compared to \$0.1 million and \$0.3 million, respectively, for the same period in 2014. As of September 30, 2015 and December 31, 2014, the Company had a receivable of \$0.1 million and \$0, respectively.

#### **NOTE 9 - LICENSE AGREEMENTS**

#### License Agreement - BioVec

On June 10, 2015, the Company and BioVec Pharma, Inc. (BioVec) entered into a license agreement (the BioVec Agreement) pursuant to which BioVec agreed to supply the Company with certain proprietary cell lines and granted to the Company a non-exclusive, worldwide license to certain of its patent rights and related know-how related to such proprietary cell lines.

As consideration for the products supplied and rights granted to the Company under the BioVec Agreement, the Company agreed to pay to BioVec an upfront fee of \$100,000 within ten business days of the effective date of the BioVec Agreement and a fee of \$300,000 within ten business days of its receipt of the first release of GMP lot of the products licensed under the BioVec Agreement. In addition, the Company

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

#### License Agreement - Leiden

On April 23, 2015, the Company and Academisch Ziekenhuis Leiden, also acting under the name Leiden University Medical Centre (Leiden), entered into a license agreement (the Leiden Agreement), pursuant to which Leiden granted to the Company an exclusive, worldwide license to its patent rights covering high affinity T-cell receptors targeting preferentially-expressed antigen in melanoma, (PRAME) and POU2AF1 epitopes. The license granted under the Leiden Agreement is subject to certain restrictions and to Leiden's retained right to use the licensed patents solely for academic research and teaching purposes, including research collaborations by Leiden with academic, non-profit research third parties; provided that Leiden provides 30 days advance written notice to the Company of such academic research collaborations.

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

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

#### **NOTE 10 - COMMITMENTS AND CONTINGENCIES**

#### Lease Agreement

On May 6, 2015, the Company entered into a lease agreement (the Lease) with Sheridan Hills Developments L.P. (the Landlord) for the lease of three spaces of approximately 25,304 square feet (the Manufacturing Space), 705 square feet (the Interior Mechanical Space) and 808 square feet (the Exterior Mechanical Space), respectively, which the Company will use to enable in-house cell therapy manufacturing. The term of the Lease began on September 1, 2015 and will continue for an initial term of five years, which may be renewed for five additional one-year periods. For the Manufacturing Space, the Company is required to remit base monthly rent of approximately \$64,841 which will increase at an average approximate rate of 3.5% each year. For the Interior Mechanical Space, the Company is required to remit base monthly rent of approximately \$1,219, which will increase at an average approximate rate of 5% each year. The monthly base rent for the Exterior Mechanical Space is approximately \$471. The Company is also required to pay additional rent in the form of its pro rata share of certain specified operating expenses of the Landlord. An early termination right is available to the Company upon certain events, including the Landlord's default on its obligations under the Lease. The newly leased spaces are located within the same building as the Company's current headquarters in Houston, Texas and are accounted for as operating leases.

#### Litigation

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

## 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, as well as our unaudited financial statements and related notes included in this Quarterly Report on Form 10-Q.

#### 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 intended 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 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 and then control components of the immune system in real time. By incorporating our CID platform, our product candidates may offer better safety and efficacy outcomes than are seen with current cellular immunotherapies.

We are developing next-generation product candidates in some of the most important areas of cellular immunotherapy, including hematopoietic stem cell transplantation, or HSCT, chimeric antigen receptor T cell therapy (CAR-Ts), and T cell receptors (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 (CARs) or T cell receptors (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", frequent transient neurologic toxicities and cases in which they have attacked healthy organs as well as the targeted tumor, sometimes resulting in death. In solid tumors, where the behavior of CAR-T cells is particularly unpredictable and results have been inconsistent, researchers are developing enhanced CAR-T cell approaches called "armored CARs" 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 of the immunotherapy cells. Each of our technologies incorporates one of these switches, for enhanced, real time control of safety and efficacy:

- *CaspaCIDe* is our safety switch, incorporated into our HSCT and in certain of our CAR-T or TCR, product candidates, where it is inactive unless the patient experiences a serious side effect. In that event, rimiducid is administered to fully or partially eliminate the cells, with the goal of terminating or attenuating the therapy and resolving the serious side effect.
- *CIDeCAR* consists of CAR-T cells modified to include the signaling domains of two proteins, MyD88 and CD40. Together, these form our proprietary dual co-stimulatory domain, MC, which is designed to activate T cells in the presence of cancer cells. Incorporation of CaspaCIDe in a CIDeCAR product candidate is intended to allow the enhanced potency of MC co-stimulation to



be deployed safely in patients.

• **GoCAR-T** consists of CAR-T cells that are modified to include the proprietary dual co-stimulatory domain, MC. In contrast to CIDeCAR, MC is structured in GoCAR-T as a rimiducid-driven molecular switch, separate from the chimeric antigen receptor. GoCAR-T is designed to allow control of the activation and proliferation of the CAR-T cells through the scheduled administration of a course of rimiducid infusions that may continue until the desired patient outcome is achieved. In the event of emergence of side effects, the level of activation of the GoCAR-T cells is designed to be attenuated by extending the interval between rimiducid doses and/or reducing the dosage per infusion.

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.** We are developing a CaspaCIDe product candidate, BPX-501, as an adjunct T-cell therapy administered after allogeneic hematopoietic stem cell transplant (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 graft-versus-host-disease (GvHD). BPX-501 is currently being evaluated in multiple Phase 1/2 clinical trials in the United States and Europe, with the initial top-line data from ongoing studies expected to be disclosed in conjunction with the Annual Meeting of the American Society of Hematology in December 2015.

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

- **BPX-401.** We are developing a CIDeCAR product candidate, BPX-401, as a next-generation CAR-T cell therapy for hematological cancers that express the CD19 antigen.
- **BPX-601.** We are developing a GoCAR-T product candidate, BPX-601, for solid tumors overexpressing prostate stem cell antigen, or PSCA, such as some pancreatic, prostate, bladder, esophageal and gastric cancers.
- **BPX-701.** We are developing a CaspaCIDe TCR product candidate, BPX-701, in collaboration with Leiden University Medical Center. BPX-701 is designed to treat solid tumors which overexpress the preferentially-expressed antigen in melanoma, or (PRAME), which include certain melanomas, sarcomas and neuroblastomas.

We expect to file Investigational New Drug Applications, (INDs) for BPX-601 and BPX-701 in the fourth quarter of 2015 and for BPX-401 in 2016. Our IND-enabling activities for each of these preclinical product candidates include manufacturing key components and developing a robust process to produce cell products that comply with regulations of the FDA, and other regulatory agencies. We have developed an efficient and scalable process to manufacture genetically modified T cells of high quality and purity. This process is currently being implemented by our third-party contract manufacturers to produce BPX-501 for our clinical trials. We expect to leverage this process as well as our resources, capabilities and expertise for the manufacture of our CAR-T and TCR product candidates. In May 2015, we entered into a lease agreement for approximately 27,000 additional square feet of space at our corporate headquarters for the manufacture of BPX-501 for clinical studies and to support the development of our expanding pipeline of TCR and CAR-T adoptive cell therapy product candidates.

#### **Critical Accounting Policies and Estimates**

The preparation of financial statements in conformity with generally accepted accounting principles requires us to make judgments, estimates and assumptions in the preparation of our 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**

#### Revenues

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

During 2011, we entered into a grant agreement with CPRIT for approximately \$5.7 million covering a three year period from July 1, 2011 through June 30, 2014. The grant initially allowed us to receive funds in advance of costs and allowable expenses being incurred. On a quarterly basis, we were required to submit a financial reporting package outlining the nature and extent of reimbursed costs under the grant. At the end of each period, any excess funds received in advance, or paid prior to reimbursement, resulted in a deferred liability or grant receivable. The CPRIT grant, under which we received payments of \$4.9 million, expired as of June 30, 2014. We recorded a grant receivable from CPRIT of \$0.3 million at December 31, 2014, which was collected during the first quarter of 2015.

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 \$1.0 million to date, of which \$0.7 million has been received. We accrue the revenue based on the costs incurred for the programs associated with the grant.

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

#### **Research and Development Expenses**

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

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

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

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

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

- per patient clinical trial costs;
- the number of patients that participate in the clinical trials;
- the number of sites included in the clinical trials;



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

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

# General and administrative expenses

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

We anticipate that our general and administrative expenses will increase in the future to support our continued research and development activities, potential commercialization of our product candidates and the increased costs of operating as a public company. These increases will likely include increased costs related to the hiring of additional personnel and fees to outside consultants, lawyers and accountants, among other expenses. Additionally, we anticipate increased costs associated with being a public company, including expenses related to services associated with maintaining compliance with NASDAQ listing rules and SEC requirements, insurance and investor relations costs.

#### Income Taxes

We did not recognize any income tax expense for the three or nine months ended September 30, 2015 or 2014.

#### **Results of Operations**

#### Comparison of the Three and Nine Months Ended September 30, 2015 and 2014

The following table sets forth our results of operations for the three and nine months ended September 30, 2015 and 2014:

	Three Months Ended September 30,						Nine Months Ended September 30,					
	2015		2014		Change		2015		2014		Change	
(in thousands)												
Grant revenues	\$	57	\$	660	\$	(603)	\$	248	\$	1,766	\$	(1,518)
Operating expenses:												
Research and development		9,792		2,257		7,535		23,522		7,881		15,641
General and administrative		3,882		1,300		2,582		8,856		2,332		6,524
Total operating expenses		13,674		3,557		10,117		32,378		10,213		22,165
Loss from operations		(13,617)		(2,897)		(10,720)		(32,130)		(8,447)		(23,683)
Other income (expense):												
Interest income		211		9		202		432		15		417
Interest expense		—		(11)		11		—		(38)		38
Change in fair value of warrant liability		—		(1,197)		1,197		—		(1,197)		1,197
Other income (expense)		(2)		—		(2)		(2)		—		(2)
Total other income (expense)		209	-	(1,199)		1,408		430		(1,220)		1,650
Net loss	\$	(13,408)	\$	(4,096)	\$	(9,312)	\$	(31,700)	\$	(9,667)	\$	(22,033)

#### Grant Revenues

Grant revenues were \$0.1 million and \$0.2 million for the three and nine months ended September 30, 2015, respectively, and \$0.7 million and \$1.8 million during the comparable periods in 2014. The decrease in grant revenues was primarily due to the expiration of our grant award from Cancer Prevention and Research Institute of Texas in June 2014.

#### Research and Development Expenses

Research and development expenses were \$23.5 million and \$7.9 million for the nine months ended September 30, 2015 and September 30, 2014, respectively. The \$15.6 million increase in research and development expenses for the nine months ended September 30, 2015, was due to an increase in clinical and manufacturing costs of \$7.4 million primarily related to BPX 501, due to increased patient enrollment in our clinical trials. The increase in research and development expenses is also due to an increase of \$1.0 million in costs related to BPX-401, \$0.5 million in costs related to BPX-601, and \$1.0 million in costs related to BPX-701, all of which are primarily related to IND enabling activities; plus the increase of \$5.8 million in general research and development costs which includes an increase of \$2.7 million in personnel costs, \$2.8 million in allocated overhead costs and \$0.3 million in other costs.

Research and development expenses were \$9.8 million and \$2.3 million for the three months ended September 30, 2015 and September 30, 2014, respectively. The \$7.5 million increase in research and development expenses for the three months ended September 30, 2015, was due to an increase in clinical and manufacturing costs of \$3.7 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 an increase of \$0.6 million in costs related to BPX-401, an increase of \$0.3 million in costs related to BPX-601, and an increase of \$0.6 million in costs related to BPX-701, all of which are primarily related to IND enabling activities; plus the increase of \$2.5 million of general research and development costs, which includes an increase of \$1.0 million in research and development personnel costs, \$1.2 million in allocated overhead costs and \$0.3 million in other costs.

#### Reclassifications

Certain research and development indirect costs, including facilities and overhead, were previously included in general and administrative costs. These research and development indirect costs are included in research and development expense in the three and nine months ended September 30, 2015, and results for the three and nine months ended September 30, 2014 have been reclassified to conform to the current year presentation. The effect of the reclassification of the results for the three and nine months ended September 30, 2014 was to increase research and development expense and reduce general and administrative expense by \$0.1 million and \$0.8 million, respectively, with no change in total operating expense or net loss.

The following table presents our research and development expense by project/category for the periods indicated (in thousands):

(in thousands)	Three Months Ended September 30,						Nine Months Ended September 30,					
Product Candidates	2015		2014		Change		2015		2014		Change	
BPX-401	\$	610	\$	—	\$	610	\$	1,030	\$		\$	1,030
BPX-501		4,294		836		3,458		9,891		2,598		7,293
BPX-601		333		—		333		525				525
BPX-701		648		_		648		1,006				1,006
General		3,907		1,421		2,486		11,070		5,283		5,787
Total	\$	9,792	\$	2,257	\$	7,535	\$	23,522	\$	7,881	\$	15,641

#### General and Administrative Expenses

General and administrative expenses were \$3.9 million and \$8.9 million, respectively, for the three and nine months ended September 30, 2015 and \$1.3 million and \$2.3 million or the three and nine months ended September 30, 2014, respectively. The increase of \$2.6 million and \$6.5 million in general and administrative expenses for the three and nine months ended September 30, 2015, respectively, was due to our overall growth and public company related costs, including an increase in personnel, legal and accounting expenses, costs related to facilities, insurance costs and travel expenses.

#### Other Income (Expense)

Other income was \$0.2 million and \$0.4 million for the three and nine months ended September 30, 2015, respectively, compared to other expense of \$1.2 million for both the three and nine months ended September 30, 2014. The change was primarily due to the recognition of a non-cash charge of \$1.2 million for the change in fair value of the warrants granted in the third quarter of 2014 in conjunction with the financing round completed in August 2014 and higher levels of interest income in 2015 periods as a result of substantially higher levels of cash and investments. Cash in excess of immediate requirements is invested in accordance with our investment policy, primarily with a view to liquidity and capital preservation.

#### 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 September 30, 2015 and December 31, 2014, we had cash, cash equivalents and investment securities of \$163.2 million and \$191.6 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.

In December 2014, we completed our initial public offering of shares of our common stock which resulted in aggregate gross proceeds to us of approximately \$160.6 million and net offering proceeds to us of approximately \$146.3 million, after deducting underwriting discounts and commissions and offering costs. Also in conjunction with our initial public offering, \$3.4 million of accrued Series B dividends were paid, of which \$0.2 million was paid in cash and the remainder was paid by issuance of 168,199 shares of our common stock.

#### Cash Flows

The following table sets forth a summary of our cash flows for the nine months ended September 30, 2015 and 2014:

	Nine Months Ended September 30,					
		2015 2014			Change	
(in thousands)						
Net cash used in operating activities	\$	(23,590)	\$	(7,158)	\$	(16,432)
Net cash used in investing activities		(75,954)		(401)		(75,553)
Net cash provided by financing activities		429		58,324		(57,895)
Net change in cash and cash equivalents	\$	(99,115)	\$	50,765	\$	(149,880)

#### **Operating Activities**

Net cash used in operating activities for the nine months ended September 30, 2015 was comprised of a net loss of \$31.7 million, which included depreciation expense of \$0.8 million and share-based compensation expense of \$5.9 million. Net cash used in operating activities was also comprised of the following primary components: an increase in receivables of \$0.1 million, an increase in prepaid expenses and other assets of \$0.7 million, and an increase in accounts payable and other liabilities of \$1.8 million.

Net cash used in operating activities for the nine months ended September 30, 2014, was comprised of a net loss of \$9.7 million, which included depreciation expense of \$0.5 million, share-based compensation expense of \$0.2 million, and loss on warrant liability of \$1.2 million. Net cash used in operating activities was also comprised of the following primary components: an increase in receivables of \$0.5 million, and an increase in accounts payable and other liabilities of \$0.9 million.

#### Investing Activities

Net cash used in investing activities for the nine months ended September 30, 2015 was \$76.0 million, consisting of the purchase of investment securities of \$79.1 million, offset by the proceeds from sale of investment securities of \$7.7 million and the purchase of property and equipment of \$4.6 million. Net cash used in investing activities for the nine months ended September 30, 2014 consisted of \$0.4 million, which was derived solely from the purchase of property and equipment.

#### Financing Activities

Net cash provided by financing activities for the nine months ended September 30, 2015 was \$0.4 million, which was derived from approximately \$0.4 million of proceeds from the exercise of stock options and purchases under the ESPP Plan. Net cash provided by financing activities for the nine months ended September 30, 2014 was \$58.3 million, which was derived from the proceeds from the issuance of preferred stock of \$62.3 million, offset by approximately \$0.2 million from the exercise of common stock warrants and \$0.1 million of proceeds from our line of credit, which were offset by payments of \$0.3 million on our line of credit.

#### **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 the uncertainty of:

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

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

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

We plan to continue to fund our operations and capital funding needs through equity and/or debt financing. We may also consider new collaborations or selectively partnering our technology. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interests of our stockholders will be diluted, and the terms may include liquidation or other preferences that adversely affect the rights of our existing stockholders. The incurrence of indebtedness would result in increased fixed payment obligations and could involve certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. If we raise

additional funds through strategic partnerships and alliances and licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies or product candidates, or grant licenses on terms unfavorable to us. Any of these actions could harm our business, results of operations and future prospects.

#### Outlook

Based on our research and development plans and our timing expectations related to the progress of our programs, we expect that our cash and cash equivalents as of September 30, 2015, which includes the net proceeds from our initial public offering, will enable us to fund our operating expenses and capital expenditure requirements through at least the first half of 2017. We have based this estimate on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we currently expect. Furthermore, our operating plan may change, and we may need additional funds to meet operational needs and capital requirements for product development and commercialization sooner than planned. Because of the numerous risks and uncertainties associated with the development and commercialization of our product candidates and the extent to which we may enter into additional collaborations with third parties to participate in their development and commercialization, we are unable to estimate the amounts of increased capital outlays and operating expenditures associated with our current and anticipated clinical trials. Our future funding requirements will depend on many factors, as we:

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

#### **Contractual Obligations and Commitments**

Our contractual obligations as of September 30, 2015 were as follows (in thousands):

	Commitment		L	ess Than 1 year	1 t	o 3 Years	3 t	to 5 Years	Ν	Aore Than 5 Years
License agreements (1)	\$	11,369	\$	3,321	\$	1,080	\$	2,545	\$	4,423
Operating lease agreements (2)		8,873		1,846		3,829		3,198		—
Contract manufacturing arrangements (3)		4,764		4,554		174		36		—
Facility lease agreement (4)		1,938		1,512		426		—		—
Sponsored research agreements (5)		359		247		112		—		_
Equipment lease agreement (6)		190		33		67		66		24
Total contractual obligations	\$	27,493	\$	11,513	\$	5,688	\$	5,845	\$	4,447

- (1) 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 clinical and regulatory milestones regarding the products covered by this license. The obligations listed in the table above represent estimates of when the milestones will be achieved. We cannot assure that the timing of the milestones will be completed when estimated or at all. See Note 9 to the unaudited financial statements included in this Quarterly Report on Form 10-Q.
- (2) Operating lease agreements The amounts above are comprised of two five-year lease agreements. The first lease will expire on January 31, 2020. See Note 13 to the audited financial statements included in our Annual Report for more information about the first lease. We entered into an additional fiveyear lease in May 2015, which began on September 1, 2015. Under this new lease, we will be responsible for monthly base rental payments which escalate on September 1st of each year until the lease expires on August 31, 2020. For more information about this second lease, see Note 10 to the unaudited financial statements included in this Quarterly Report on Form 10-Q.
- (3) Contract manufacturing arrangements We have entered into several manufacturing service arrangements with various terms. The obligations listed in the table above represent estimates of when certain services will be performed. See Note 8 to the audited financial statements included in our Annual Report.

- (4) Facility lease agreement In March 2013 we entered into a two-year manufacturing facility agreement for cell processing for a clinical trial. In February 2015, the agreement was extended for an additional two years. In September 2015, we amended a previous manufacturing services agreement for cell processing. The amended agreement expires in December 2016.
- (5) Sponsored research agreements We have entered into two separate sponsored research agreements to undertake research which is of mutual interest to all parties. One agreement includes a commitment over 14 months and the other includes a commitment over a three-year period.
- (6) Equipment lease agreement We have entered into an office equipment lease agreement covering a six year term. The commitment includes equipment, maintenance and supplies.

#### **Recent Accounting Pronouncements**

There are no recent accounting pronouncements that have a material impact on our financial statements.

#### **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 objectives of our investment activities are 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 September 30, 2015, we had cash, cash equivalents and investment securities of \$163.2 million. Our cash, cash equivalents and 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 September 30, 2015 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 September 30, 2015, 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

We maintain disclosure controls and procedures that are designed to ensure that information required to be disclosed in the reports that we file or submit under the Securities and Exchange Act of 1934 is (1) recorded, processed, summarized, and reported within the time periods specified in the SEC's rules and forms, and (2) accumulated and communicated to our management, including our principal executive officer and principal financial officer, to allow timely decisions regarding required disclosure.

As of September 30, 2015, our management, with the participation of our principal executive officer and principal financial officer, evaluated the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Securities and Exchange Act of 1934). Our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives, and management necessarily applies its judgment in evaluating the cost-benefit relationship

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of possible controls and procedures. Our principal executive officer and principal financial officer have concluded, based upon the evaluation described above, that as of September 30, 2015 our disclosure controls and procedures were effective at the reasonable assurance level.

#### **Changes in Internal Control over Financial Reporting**

During the nine months ended September 30, 2015, we implemented changes to our internal control procedures over financial reporting to remediate our previously reported material weaknesses in our Annual Report. We hired additional personnel, including a chief financial officer and other senior finance executives, and consultants to augment our accounting staff, as well as implemented additional, formalized policies and procedures related to accounting and financial reporting, particularly surrounding non-routine transactional and financial reporting. These policies and procedures are followed by all accounting personnel.

Other than those discussed above, there have been no other changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Securities Exchange Act of 1934, as amended) during our most recent fiscal quarter that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting. Our process for evaluating controls and procedures is continuous and encompasses constant improvement of the design and effectiveness of established controls and procedures and the remediation of any deficiencies which may be identified during this process.

#### PART II. OTHER INFORMATION

#### **Item 1. Legal Proceedings**

None.

#### Item 1A. Risk Factors

Investing in our common stock is 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, our Quarterly Report on Form 10-Q for the quarter ended June 30, 2015, and in other reports we file with the SEC.

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

None.

## **Purchase of Equity Securities**

We did not purchase any of our registered securities during the period covered by this Quarterly Report on Form 10-Q.

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

On December 17, 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.

As of September 30, 2015, we have used the net offering proceeds from our initial public offering to fund operations, capital expenditures, working capital and other general corporate purposes and for debt repayment. We are holding the balance of the net proceeds from the offering in cash, cash equivalents and investment securities. There has been no material change in our planned use of the balance of the net proceeds from the offering described in our final prospectus filed with the SEC on December 17, 2014 pursuant to Rule 424(b) under the Securities Act.

#### Item 6. Exhibits

The exhibits filed as part of this Quarterly Report on Form 10-Q are set forth on the Exhibit Index, which is incorporated herein by reference.

### Signatures

Pursuant to the requirements of 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.

By: /s/ Thomas J. Farrell

Thomas J. Farrell President and Chief Executive Officer

By: /s/ Alan A. Musso

Alan A. Musso Chief Financial Officer and Treasurer Principal Financial and Accounting Officer

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Date: November 9, 2015

Date: November 9, 2015

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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(2)	Warrant to Purchase Common Stock issued to the State of Texas, dated September 27, 2007.
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.

# **CERTIFICATION PURSUANT TO**

# RULE 13a-14(a) and 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED, AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Thomas J. Farrell, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Bellicum Pharmaceuticals, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

- a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
- b) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation;
- c) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

- a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
- b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 9, 2015

By:

Thomas J. Farrell President and Chief Executive Officer

/s/ Thomas J. Farrell

## **CERTIFICATION PURSUANT TO**

# RULE 13a-14(a) and 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED, AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Alan A. Musso, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Bellicum Pharmaceuticals, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

- a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
- b) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation;
- c) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

- a) All significant deficiencies and material weaknesses in the design or operation of internal controls over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
- b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 9, 2015

By:

Alan A. Musso Chief Financial Officer and Treasurer (Principal Financial and Accounting Officer)

/s/ Alan A. Musso

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

In connection with the Quarterly Report on Form 10-Q for the quarter ended June 30, 2015 (the "Report") of Bellicum Pharmaceuticals, Inc. (the "Registrant"), as filed with the Securities and Exchange Commission on the date hereof, the undersigned, in their capacities as officers of the Registrant, do each hereby certify, that, to the best of such officer's knowledge:

- (1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and
- (2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Registrant.

/s/ Thomas J. Farrell Thomas J. Farrell President and Chief Executive Officer (Principal Executive Officer) November 9, 2015

/s/ Alan A. Musso

Alan A. Musso Chief Financial Officer and Treasurer (Principal Financial and Accounting Officer) November 9, 2015