

BELLICUM PHARMACEUTICALS, INC

Form 10-Q

November 06, 2018

Table of Contents

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 September 30, 2018

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 every Interactive Data File required to be submitted 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 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 filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act. (Check one):

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

Large accelerated filer       Accelerated filer                       x  
Non-accelerated filer         Smaller reporting company  o  
Emerging growth company  x

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.  x

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

Yes  No  x

As of October 31, 2018, there were 43,361,159 outstanding shares of Bellicum's common stock, par value, \$0.01 per share.

---

TABLE OF CONTENTS

	Page
<u>PART I. FINANCIAL INFORMATION</u>	<u>3</u>
<u>Item 1. Condensed Consolidated Financial Statements (Unaudited) Condensed Consolidated Balance Sheets as of September 30, 2018 (Unaudited) and December 31, 2017 Condensed Consolidated Statements of Operations and Comprehensive Income (Loss) for the three and nine months ended September 30, 2018 and 2017 (Unaudited) Condensed Consolidated Statements of Cash Flows for the nine months ended September 30, 2018 and 2017 (Unaudited) Notes to Condensed Consolidated Financial Statements (Unaudited)</u>	3
<u>Item 2. Management's Discussion and Analysis of</u>	17

	<u>Financial Condition and Results of Operations Quantitative and Qualitative</u>	
<u>Item 3.</u>	<u>Disclosures About Market Risk</u>	<u>26</u>
<u>Item 4.</u>	<u>Controls and Procedures</u>	<u>27</u>
	<u>PART II. OTHER INFORMATION</u>	<u>28</u>
<u>Item 1.</u>	<u>Legal Proceedings</u>	<u>28</u>
<u>Item 1A.</u>	<u>Risk Factors Unregistered</u>	<u>28</u>
<u>Item 2.</u>	<u>Sales of Equity Securities and Use of Proceeds</u>	<u>60</u>
<u>Item 6.</u>	<u>Exhibits</u>	<u>61</u>
	<u>SIGNATURES</u>	<u>63</u>

## PART I. FINANCIAL INFORMATION

## Item 1. Financial Statements

Bellicum Pharmaceuticals, Inc.

Condensed Consolidated Balance Sheets

(In thousands, except share and par value amounts)

	September 30, 2018	December 31, 2017
	(Unaudited)	
<b>ASSETS</b>		
Current assets:		
Cash and cash equivalents	\$ 53,027	\$ 38,839
Investment securities, available for sale - short-term	59,720	60,057
Accounts receivable, interest and other receivables	538	320
Prepaid expenses and other current assets	2,254	2,434
Total current assets	115,539	101,650
Investment securities, available for sale - long-term	—	1,368
Property and equipment, net	22,402	25,942
Restricted cash	5,635	6,190
Other assets	396	378
<b>TOTAL ASSETS</b>	<b>\$ 143,972</b>	<b>\$ 135,528</b>
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Current liabilities:		
Accounts payable	\$ 1,843	\$ 3,287
Accrued expenses and other current liabilities	8,837	6,392
Current portion of capital lease obligations	37	31
Current portion of deferred revenue	3,295	2,049
Current portion of deferred rent	411	397
Total current liabilities	14,423	12,156
Long-term liabilities:		
Long-term debt, net of deferred financing costs	35,609	34,946
Capital lease obligations	102	131
Deferred revenue	—	2,054
Deferred rent	1,381	1,593
<b>TOTAL LIABILITIES</b>	<b>51,515</b>	<b>50,880</b>
Commitments and contingencies: (Note 12)		
Stockholders' equity:		
Preferred stock: \$0.01 par value; 10,000,000 shares authorized: no shares issued and outstanding	—	—
Common stock, \$0.01 par value; 200,000,000 shares authorized at September 30, 2018 and December 31, 2017, 44,028,622 shares issued and 43,351,159 shares outstanding at September 30, 2018; 33,962,640 shares issued and 33,285,177 shares outstanding at December 31, 2017	440	340
Treasury stock: 677,463 shares held at September 30, 2018 and December 31, 2017	(5,056 )	(5,056 )
Additional paid-in capital	490,436	411,922
Accumulated other comprehensive loss	(35 )	(46 )
Accumulated deficit	(393,328 )	(322,512 )
Total stockholders' equity	92,457	84,648

TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY	\$ 143,972	\$ 135,528
--	------------	------------

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

3

---

Bellicum Pharmaceuticals, Inc.

Condensed Consolidated Statements of Operations and Comprehensive Loss

(In thousands, except share and per share amounts)

(Unaudited)

	Three months ended		Nine months ended	
	September 30,		September 30,	
	2018	2017	2018	2017
REVENUES				
Grants	\$292	\$126	\$808	\$254
Total revenues	292	126	808	254
OPERATING EXPENSES				
Research and development	16,413	18,101	51,361	51,355
License fees	139	151	319	849
General and administrative	6,968	4,579	18,027	15,992
Total operating expenses	23,520	22,831	69,707	68,196
Loss from operations	(23,228 )	(22,705 )	(68,899 )	(67,942 )
OTHER INCOME (EXPENSE):				
Interest income	492	284	1,196	788
Interest expense	(1,065 )	(1,010 )	(3,113 )	(2,707 )
Total other expense	(573 )	(726 )	(1,917 )	(1,919 )
NET LOSS	\$(23,801)	\$(23,431)	\$(70,816)	\$(69,861)
Net loss per common share attributable to common shareholders, basic and diluted	\$(0.55 )	\$(0.71 )	\$(1.81 )	\$(2.24 )
Weighted-average shares outstanding, basic and diluted	43,334,727	33,178,611	39,168,559	31,204,521
Net loss	\$(23,801)	\$(23,431)	\$(70,816)	\$(69,861)
Other comprehensive income (loss):				
Unrealized gain (loss) on investment securities	33	22	11	(1 )
Comprehensive loss	\$(23,768)	\$(23,409)	\$(70,805)	\$(69,862)

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)

	Nine months ended September 30,	
	2018	2017
<b>CASH FLOWS FROM OPERATING ACTIVITIES:</b>		
Net loss	\$(70,816)	\$(69,861)
Adjustments to reconcile net loss to net cash used in operating activities:		
Share-based compensation	10,854	10,217
Depreciation expense	4,916	2,524
Amortization of premium on investment securities, net	118	214
Amortization of lease liability	(198)	(71)
Amortization of deferred financing costs	663	604
Changes in operating assets and liabilities:		
Receivables	(218)	(134)
Prepaid expenses and other assets	162	(685)
Accounts payable	(1,444)	(923)
Accrued liabilities and other	2,390	(2,046)
Deferred revenue	(808)	—
<b>NET CASH USED IN OPERATING ACTIVITIES</b>	<b>(54,381)</b>	<b>(60,161)</b>
<b>CASH FLOWS FROM INVESTING ACTIVITIES:</b>		
Purchases of investment securities	(50,641)	(36,283)
Proceeds from sale of investment securities	52,239	45,536
Purchases of property and equipment	(1,321)	(10,554)
<b>NET CASH PROVIDED BY (USED IN) INVESTING ACTIVITIES</b>	<b>277</b>	<b>(1,301)</b>
<b>CASH FLOWS FROM FINANCING ACTIVITIES:</b>		
Proceeds from stock offering, net of offering costs	64,665	64,568
Proceeds from exercise of stock options	2,996	1,453
Proceeds from issuance of common stock - ESPP	99	167
Proceeds from notes payable	—	10,000
Payment of debt issuance costs	—	(75)
Payment on capital lease obligations	(23)	(16)
<b>NET CASH PROVIDED BY FINANCING ACTIVITIES</b>	<b>67,737</b>	<b>76,097</b>
<b>NET CHANGE IN CASH, CASH EQUIVALENTS AND RESTRICTED CASH</b>	<b>13,633</b>	<b>14,635</b>
<b>CASH, CASH EQUIVALENTS AND RESTRICTED CASH AT BEGINNING OF PERIOD</b>	<b>45,029</b>	<b>42,780</b>
<b>CASH, CASH EQUIVALENTS AND RESTRICTED CASH AT END OF PERIOD</b>	<b>\$58,662</b>	<b>\$57,415</b>
<b>SUPPLEMENTAL CASH FLOW INFORMATION:</b>		
Interest paid	\$2,451	\$2,014
<b>NON-CASH INVESTING AND FINANCING ACTIVITIES:</b>		
Purchases of property and equipment in accounts payables and accrued liabilities	\$55	\$1,695
Accrued debt issuance costs	\$—	\$695
Capital lease obligations incurred for equipment	\$—	\$23

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., (“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 CAR T, TCR, and hematopoietic stem cell transplantation.

In 2017, Bellicum formed two wholly-owned subsidiaries, Bellicum Pharma Limited, a private limited company organized under the laws of the United Kingdom, and Bellicum Europe GmbH, a private limited liability company organized under Swiss law, for the purpose of developing and commercializing product candidates in Europe. Bellicum, Bellicum Pharma Limited and Bellicum Europe GmbH are collectively referred to herein as the “Company”.

**NOTE 2 - BASIS OF PRESENTATION**

The accompanying interim condensed consolidated financial statements are unaudited. These unaudited interim condensed consolidated 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, 2017 (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 obtain regulatory approval and commercialize any of its product candidates, the Company will not be able to generate product revenue or achieve profitability. As of September 30, 2018, the Company had an accumulated deficit of \$393.3 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 subsidiaries, neither of which have had any material activity to date. All significant intercompany balances and transactions have been eliminated in consolidation.

#### Revenue Recognition

The Company has not yet generated any revenue from product sales. The Company's source of revenue for the three and nine months ended September 30, 2018 and 2017 has been from grants. When grant funds are received after costs have been incurred, the Company accrues revenue and records a grant receivable. Cash received from grants in advance of incurring qualifying costs is recorded as deferred revenue and recognized as revenue when qualifying costs are 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.

#### 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 in fair value is determined to be other than temporary, the Company records an impairment charge in the statement of operations and comprehensive loss and establishes a new cost basis in the investment.

#### Property and Equipment

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

#### 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.

#### Rent and Deferred Rent

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

#### Fair Value of Financial Instruments

Accounting standards include disclosure requirements around fair values used for certain financial instruments and establish a fair value hierarchy. The three-tier hierarchy prioritizes valuation inputs into three levels based on the extent to which inputs used in measuring fair value are observable in the market, as described further in Note 5. The Company believes the recorded values of its financial instruments, including cash and cash equivalents, accounts payable and accrued liabilities approximate their fair values due to the short-term nature of these instruments.

#### Financial Instruments and Credit Risks

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

#### 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. 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.

### Comprehensive Loss

Comprehensive income (loss) is defined as the change in equity of a business enterprise during a period, from transactions, and other events and circumstances from non-owner sources. Components of comprehensive income (loss) includes, among other items, unrealized gains and losses on the changes in fair value of investments. These components are added, net of their related tax effect, to the reported net income (loss) to arrive at comprehensive income (loss). The components of accumulated other comprehensive loss at September 30, 2018 and December 31, 2017, on the Company's balance sheet was comprised of the net unrealized holding gains and losses on the Company's investment securities. See Note 5 for further detail of the unrealized holding gains and losses on the Company's investment securities.

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

Basic net loss per share attributable to common stockholders is calculated by dividing the net loss attributable to common stockholders by the weighted-average number of 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 September 30,	
	2018	2017
Common Stock Equivalents:	Number of shares	
Options to purchase common stock	5,868,433	5,299,158
Unvested shares of restricted stock units	205,155	96,250
Unvested shares of restricted stock	14,707	44,119
Total common stock equivalents	6,088,295	5,439,527

### New Accounting Requirements and Disclosures

In February 2016, the FASB issued ASU 2016-02, "Leases", which established new ASC Topic 842 ("ASC 842"), to increase transparency and comparability among organizations by recognizing lease assets and lease liabilities on the balance sheet and disclosing key information about leasing arrangements. Under the new guidance, a lessee will be required to recognize assets and liabilities for leases with lease terms of more than 12 months. Consistent with current GAAP, the recognition, measurement, and presentation of expenses and cash flows arising from a lease by a lessee primarily will depend on its classification as a finance or operating lease. However, unlike current GAAP which requires only capital leases to be recognized on the balance sheet, the new standard will require both types of leases to be recognized on the balance sheet. ASC 842 also will require disclosures to help investors and other financial statement users better understand the amount, timing, and uncertainty of cash flows arising from leases. These disclosures include qualitative and quantitative requirements, providing additional information about the amounts recorded in the financial statements.

ASC 842 was previously required to be adopted using the modified retrospective approach. However, in July 2018, the FASB issued ASU 2018-11, which allows for retrospective application with the recognition of a cumulative-effect adjustment to the opening balance of retained earnings in the period of adoption. Under this option, entities would not

need to apply ASC 842 (along with its disclosure requirements) to the comparative prior periods presented.

9

---

ASC 842 is effective for the Company in the first quarter of 2019 and management expects that most of its operating leases (primarily office space) will be recognized as operating lease liabilities and right of use assets on its balance sheet. Management is continuing to evaluate the impact that the adoption of this standard will have on the consolidated financial statements but currently believe it is likely that the Company will elect to adopt certain of the optional practical expedients, including the package of practical expedients, which, among other things, gives the option to not reassess: 1) whether expired or existing contracts are or contain leases; 2) the lease classification for expired or existing leases; and 3) initial direct costs for existing leases.

In 2018, the FASB issued ASU No. 2018-07, “Compensation - Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting”, which changes the measurement date for share-based awards to the grant date, instead of the previous requirement to remeasure the awards through the performance completion date. ASU No. 2018-07 is effective for the Company for fiscal years beginning after December 31, 2018, including interim periods within that fiscal year. Early adoption is permitted. The Company does not believe adopting ASU No. 2018-07 will have a material impact on its consolidated financial statements.

In 2018, the FASB issued ASU No. 2018-13, “Fair Value Measurement (Topic 820): Disclosure Framework—Changes to the Disclosure Requirements for Fair Value Measurement”, which modifies fair value disclosures and removes some disclosure requirements for both public and private companies. In addition, public companies are subject to some new disclosure requirements which requires to disclose the changes in unrealized gains and losses for the period included in other comprehensive income for recurring Level 3 fair value measurements held at the end of the reporting period and the range and weighted average of significant unobservable inputs used to develop Level 3 fair value measurements. ASU No. 2018-13 is effective for all entities for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2019. Early adoption is permitted. The Company does not believe adopting ASU No. 2018-13 will have a material impact on its consolidated financial statements.

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

As of September 30, 2018, and December 31, 2017, respectively, the Company maintained \$5.6 million and \$6.2 million as restricted cash.

During 2017, the Company received \$4.2 million from the Cancer Prevention and Research Institute of Texas, or “CPRIT”, which is being held in a separate account to be used for costs solely related to the CPRIT grant. Release of the CPRIT funds are subject to the terms of the grant agreement and requirements therein and require the authorization of CPRIT. During the nine months ended September 30, 2018, CPRIT authorized the release of \$88,000 of restricted funds from the CPRIT account, leaving a balance of \$4.1 million at September 30, 2018. No funds were released during the three months ended September 30, 2018. For more information about the CPRIT grant, see Note 9.

The remaining \$1.5 million of restricted cash as of September 30, 2018 and the \$2.0 million in 2017 is held in escrow to cover specific construction of manufacturing improvement costs related to the facility lease. The release of the escrowed funds is subject to the terms of the escrow agreement and requirements therein including approval by both the Company and the landlord based on authorized completion of certain aspects of the manufacturing 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.

	September 30, 2018	December 31, 2017
	(in thousands)	
Cash and cash equivalents <sup>(1)</sup>	\$53,027	\$ 38,839
Restricted cash, noncurrent	5,635	6,190
Total cash, cash equivalents and restricted cash shown in the statements of cash flows	\$58,662	\$ 45,029

<sup>(1)</sup> As of September 30, 2018, and December 31, 2017, the Company invested approximately \$37.1 million and \$25.6 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 September 30, 2018 and December 31, 2017:

	Fair Value Measurements at Reporting Date			
	Balance at September 30, 2018	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	\$37,085	\$37,085	\$ —	\$ —
Total Cash Equivalents	\$37,085	\$37,085	\$ —	\$ —
Investment Securities:				
U.S. government agency-backed securities	\$15,930	\$—	\$ 15,930	\$ —
Corporate debt securities	43,790	—	43,790	—
Total Investment Securities	\$59,720	\$—	\$ 59,720	\$ —

	Fair Value Measurements at Reporting Date			
	Quoted prices in active			
	Balance at December 31, 2017	markets for identical assets (Level 1)	Significant observable inputs (Level 2)	Significant unobservable inputs (Level 3)
	(in thousands)			
Cash Equivalents:				
Money market funds	\$25,550	\$25,550	\$ —	\$ —
Total Cash Equivalents	\$25,550	\$25,550	\$ —	\$ —
Investment Securities:				
U.S. government agency-backed securities	\$22,604	\$ —	\$ 22,604	\$ —
Corporate debt securities	38,821	—	38,821	—
Total Investment Securities	\$61,425	\$ —	\$ 61,425	\$ —

U.S. Treasury, U.S. government agency-backed securities and corporate debt securities 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, 2018 and December 31, 2017:

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Aggregate Estimated Fair Value
September 30, 2018	(in thousands)			
Investment Securities:				
U.S. government agency-backed securities	\$15,944	\$ —	\$ (14 )	\$ 15,930
Corporate debt securities	43,811	1	(22 )	43,790
Total Investment Securities	\$59,755	\$ 1	\$ (36 )	\$ 59,720
December 31, 2017	(in thousands)			
U.S. government agency-backed securities	\$22,632	\$ —	\$ (28 )	\$ 22,604
Corporate debt securities	38,839	13	(31 )	38,821
Total	\$61,471	\$ 13	\$ (59 )	\$ 61,425

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

Table of Contents

## NOTE 6 - PROPERTY AND EQUIPMENT

Property and equipment consists of the following:

		September 30, 2018	December 31, 2017
	Estimated Useful Lives (in thousands)		
Leasehold improvements	5 Years	\$21,633	\$ 21,462
Lab equipment	5 Years	8,373	7,766
Office furniture	5 Years	1,702	1,701
Manufacturing equipment	5 Years	1,891	1,815
Computer and office equipment	3 to 5 Years	1,431	1,074
Equipment held under capital leases	5 Years	204	204
Software	3 Years	378	216
Total		35,612	34,238
Less: accumulated depreciation		(13,210 )	(8,296 )
Property and equipment, net		\$22,402	\$ 25,942

During the nine months ended September 30, 2018 and 2017, the Company recorded \$4.9 million and \$2.5 million of depreciation expense, respectively. Leasehold improvements as of September 30, 2018 and December 31, 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:

	September 30, 2018	December 31, 2017
	(in thousands)	
Accrued construction costs	\$457	\$ 565
Accrued payroll	2,619	2,682
Accrued patient treatment costs	2,104	1,392
Accrued sponsored research	937	85
Accrued manufacturing costs	340	370
Accrued other	2,380	1,298
Total accrued expenses and other current liabilities	\$8,837	\$ 6,392

## NOTE 8 - DEBT

## Hercules Loan

On March 10, 2016, the Company, entered into a Loan and Security Agreement with Hercules Capital, Inc. Hercules Technology II, L.P., and Hercules Technology III, L.P., or collectively, Hercules, as a lender, under which the Company borrowed \$15.0 million. The Company borrowed an additional \$5.0 million and \$10.0 million on September 15, 2016 and March 8, 2017, respectively. The total debt was 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 intellectual property. The Company paid expenses related to the loan of \$0.3 million which, along with a final facility charge of \$2.1 million was recorded as deferred financing costs. Interest expense in the three and nine months ended September 30, 2017 included \$0.2 million and \$0.6 million, respectively, of amortized deferred financing costs. For additional information about the Hercules Loan Agreement, see Note 8 to the audited financial statements in the Annual Report.

On December 21, 2017, the Company repaid the outstanding balance, accrued interest and final facility charges totaling \$32.9 million, which included a prepayment charge of \$0.6 million with proceeds from a new loan from

Oxford Finance, LLC, discussed below.

#### Oxford Loan

On December 21, 2017 (the “Oxford Closing Date”), the Company entered into a loan and security agreement (the “Oxford Loan Agreement”) with Oxford Finance LLC, as the collateral agent and a lender, pursuant to which the Company borrowed \$35.0 million in a single term loan (the “Oxford Loan”) on the Oxford Closing Date. As discussed above, on the Oxford Closing Date, the Company used approximately \$32.9 million of the proceeds from the Oxford Loan to repay its indebtedness to Hercules. For additional information about the Oxford Loan Agreement, see Note 8 to the audited financial statements contained in the Annual Report.

The Company paid expenses related to the Oxford Loan Agreement of \$0.1 million, which, along with the final facility charge of \$3.0 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 nine-month periods ended September 30, 2018, interest expense included \$0.3 million and \$0.7 million, 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 - GRANT REVENUE

##### Cancer Research Grant Contract

During 2017, the Company entered into a Cancer Research Grant Contract (the “Agreement”) with CPRIT, pursuant to which CPRIT awarded a grant (the “Award”) of approximately \$16.9 million to the Company to fund development of rivo-cel for hematologic cancer. The Award is contingent upon funds being available during the term of the Agreement and subject to CPRIT's ability to perform its obligations under the Agreement. For additional information about the Agreement, see Note 9 to the audited financial statements in the Annual Report.

During 2017, the Company received \$4.2 million in advance funding from CPRIT, which was recorded as deferred revenue. During the three and nine-month periods ended September 30, 2018, the Company recognized expenses and accrued revenue of \$0.3 million and \$0.8 million, respectively for work performed under the CPRIT grant.

##### NIH Grant

During 2013, the Company entered into a grant agreement with the National Institute of Health, or NIH. The grant was a modular multi-year grant with funds being awarded each year based on the progress of the program being funded. The Company recorded grant revenue of \$0.1 million in the nine months ended September 30, 2017. The grant expired on March 31, 2017.

#### NOTE 10 - STOCKHOLDERS' EQUITY

## Table of Contents

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. 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 April 20, 2018, the Company completed an underwritten public offering of 9,200,000 shares of its common stock at a price of \$7.50 per share, for an aggregate offering size of \$69.0 million, pursuant to a registration statement on Form S-3. The net proceeds to the Company, after deducting underwriting discounts, and commissions and offering expenses was approximately \$64.7 million. These costs have been recorded as a reduction of the proceeds received from the offering.

### NOTE 11 - SHARE-BASED COMPENSATION PLANS

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

At September 30, 2018, 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 incentive and non-qualified stock options to employees, including officers, non-employee directors and consultants to the Company. As of September 30, 2018, there were 96,293 shares of common stock reserved for issuance pursuant to outstanding options 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 September 30, 2018, there were 825,828 shares of common stock reserved for issuance pursuant to outstanding options granted under the 2011 Plan. The 2011 Plan replaced the 2006 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 IPO. The 2014 Plan provides for the issuance of equity awards, including incentive and non-qualified stock options and restricted stock awards to employees, including officers, non-employee directors and consultants to the Company or its affiliates. The 2014 Plan also provides for the grant of performance cash awards and performance-based stock awards.

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 September 30, 2018, there were 5,166,174 outstanding awards, comprised of 4,021,312 options, 925,000 inducement option awards, 14,707 shares of restricted stock, 51,250 inducement restricted stock units and 153,905 restricted stock units outstanding. There were 1,900,685 shares available for issuance under the 2014 Plan at September 30, 2018.

2014 Employee Stock Purchase Plan

The 2014 Employee Stock Purchase Plan (the “ESPP”) provides for eligible Company employees, as defined by the ESPP, to be given an opportunity to purchase the Company's common stock at a discount, through payroll deductions, with stock purchases being made upon defined purchase dates. The ESPP authorizes the issuance of up to 550,000 shares of the Company’s common stock to participating employees, and allows eligible employees to purchase shares of common stock at a 15% discount from the lesser of the grant date or purchase date fair market value. There were 13,779 and 19,204 shares purchased by the ESPP in the nine-month periods ended September 30, 2018 and 2017, respectively. There were no ESPP purchases in the three-month periods ended September 30, 2018 and 2017. As of September 30, 2018, there were 446,248 shares available for issuance under the ESPP.

	Nine months ended September 30, 2018 2017 (amounts in thousands)	
A summary of activity within the ESPP follows:		
Deductions from employees	\$ 168	\$ 226
Share-based compensation expense recognized	\$ 111	\$ 183
Remaining share-based compensation expense	\$ 285	\$ 311

### 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 has been estimated, with the following weighted-average assumptions:

	Nine months ended September 30, 2018 2017	
Risk-free interest rate	2.63 %	2.06 %
Volatility	72.1 %	71.7 %
Expected life (years)	6.08	6.08
Expected dividend yield	— %	— %

Share-based compensation expense by classification for the nine months ended September 30, 2018 and 2017 are as follows:

	Three Months Ended September 30, 2018 2017 (in thousands)		Nine Months Ended September 30, 2018 2017 (in thousands)	
Research and development	\$ 1,606	\$ 1,802	\$ 4,898	\$ 4,870
General and administrative	2,070	1,861	5,956	5,347
Total	\$ 3,676	\$ 3,663	\$ 10,854	\$ 10,217

At September 30, 2018, total compensation cost not yet recognized was \$17.6 million and the weighted-average period over which this amount is expected to be recognized is 2.46 years.

The following table summarizes the stock option activity for all stock plans during the nine months ended September 30, 2018:

	Options and Inducement awards	Weighted- Average Exercise Price Per Share	(in years) Weighted- Average Contractual Life	Aggregate Intrinsic Value <sup>(1)</sup> (in thousands)
Outstanding at December 31, 2017	5,286,472	\$ 12.35	7.35	\$ 7,223
Granted <sup>(2)</sup>	1,965,191	\$ 7.83		
Exercised	(837,392 )	\$ 3.58		\$ 4,225
Forfeited	(545,838 )	\$ 16.27		
Outstanding at September 30, 2018	5,868,433	\$ 11.72	7.80	\$ 1,794
Exercisable at September 30, 2018	2,929,150	\$ 13.62	6.55	\$ 1,658



(1) 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 December 31, 2017 and September 30, 2018.

(2) Includes 295,000 of inducement option awards granted in 2018.

The following table summarizes the stock award activity for all stock plans during the nine months ended September 30, 2018:

	Restricted Stock Awards and Units	Aggregate Intrinsic Value <sup>(1)</sup> (in thousands)
December 31, 2017 <sup>(2)</sup>	140,663	\$ 1,183
Granted <sup>(3)</sup>	161,250	
Vested	(38,770 )	\$ 342
Forfeited	(43,281 )	
Outstanding at September 30, 2018	219,862	\$ 1,354

(1) The aggregate intrinsic value is calculated as the fair value of restricted stock and restricted stock units at December 31, 2017 and September 30, 2018.

(2) At December 31, 2017, there were 29,413 shares of restricted common stock and 111,250 restricted stock units outstanding.

(3) Includes 40,000 of inducement restricted stock units granted during 2018.

#### NOTE 12 - COMMITMENTS AND CONTINGENCIES

##### Litigation

On February 6, 2018, a purported securities class action complaint captioned Nipun Kakkar v. Bellicum Pharmaceuticals, Inc., Rick Fair and Alan Musso was filed against the Company, and certain of its officers in the U.S. District Court for the Southern District of Texas, Houston Division. A second substantially similar class action was filed on March 14, 2018 by plaintiff Frances Rudy against the same defendants in the same court. The lawsuits purport to assert class action claims on behalf of purchasers of the Company's securities during the period from May 8, 2017 through January 30, 2018. The complaints allege that the defendants violated the Securities Exchange Act of 1934, as amended (the "Exchange Act"), by making materially false and misleading statements concerning the Company's clinical trials being conducted in the U.S. to assess rivo-cel (rivogenlecleucel, formerly known as BPX-501) as an adjunct T-cell therapy administered after allogeneic hematopoietic stem cell transplantation. The complaints purport to assert claims for violations of Sections 10(b) and 20(a) of the Exchange Act and Rule 10b-5 promulgated thereunder. The complaints seek, on behalf of the purported class, an unspecified amount of monetary damages, interest, fees and expenses of attorneys and experts, and other relief. On April 9, 2018, the District Court consolidated the two lawsuits under the Kakkar action and motions were filed by putative class members for appointment as lead plaintiff and approval of lead counsel. The District Court has yet to rule on the motions. On July 19, 2018, a purported shareholder derivative complaint captioned Seung Paik v. Richard A. Fair, et al. was filed against the Company's directors and certain of the Company's officers in the U.S. District Court for the Southern District of Texas, Houston Division. The lawsuit purports to seek damages on behalf of the Company against the individual defendants for breach of fiduciary duty, waste, unjust enrichment and violations of Section 14(a) of the Exchange Act. The complaint alleges that the defendants caused or allowed the Company to disseminate misstatements regarding the clinical trials for rivo-cel and to make false or misleading statements in the proxy materials for the Company's 2017 annual meeting of stockholders. On October 3, 2018, the District Court granted the Company's motion to stay the derivative cause of action until reinstated on motion of the parties.

#### NOTE 13 - SUBSEQUENT EVENTS

On October 5, 2018, the Company entered into an Open Market Sale Agreement<sup>SM</sup> with Jefferies LLC, as sales agent, pursuant to which the Company may offer and sell, from time to time, through Jefferies, shares of the Company's common stock having an aggregate offering price of up to \$60.0 million. The shares will be offered and sold pursuant to the Company's shelf registration statement on Form S-3.

## Table of Contents

The Company will pay Jefferies LLC a commission of up to 3.0% of the aggregate gross proceeds from each sale of shares, reimburse certain legal fees and disbursements and provide Jefferies LLC with customary indemnification and contribution rights. The Sales Agreement may be terminated by Jefferies LLC or the Company at any time upon notice to the other party.

### 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, 2017, filed with the SEC on March 13, 2018, 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 this Quarterly Report on Form 10-Q, 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 chimeric antigen receptor T cell therapy, or CAR T, T cell receptors, or TCRs and hematopoietic stem cell transplantation, or HSCT. 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 CAR T cells 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 efficacy results have been poor, researchers are developing enhanced CAR T cell approaches that raise even greater safety concerns. 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 or cancer recurrence due to the lack of an effective immune system following a transplant.

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™ (also known as inducible Caspase-9, or iC9) is our safety switch, incorporated into our HSCT and TCR product candidates, and into academic CAR T collaborations, where it is inactive unless the patient experiences a serious side effect. In that event, rimiducid is administered to induce Caspase-9 and eliminate a majority of the cells, with the goal of attenuating the therapy and resolving the serious side effect.

Our activation switch (also known as inducible MyD88/CD40, or iMC) incorporated into our GoCAR-T™ product candidates is designed to enable 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” GoCAR-T that is designed to provide a user-controlled system for managing proliferation and/or 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 clinical product candidates are described below.

Rivo-cel (rivogencleleucel, formerly known as BPX-501) is a CaspaCIDE product candidate designed as an adjunct polyclonal T cell therapy administered after allogeneic HSCT. Rivo-cel is designed to improve transplant outcomes by enhancing the recovery of the immune system following an HSCT procedure. Rivo-cel 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 rivo-cel for treatment in HSCT, and for activator agent rimiducid for the treatment of GvHD. Additionally, rivo-cel 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.

Based on interactions with the European Medicines Agency, or the EMA, we believe that data from the European arm of our BP-004 trial could form the basis of Marketing Authorisation Applications, or MAAs, for rivo-cel and rimiducid for pediatric patients with certain orphan inherited blood disorders or treatment-refractory hematological cancers. 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. In place of a randomized trial, we are collecting data from the C-004 study, a concurrent observational study in pediatric patients receiving a matched unrelated donor hematopoietic stem cell transplant. We expect to report updated results from the BP-004 clinical trial in the fourth quarter of 2018 and to file MAAs for European marketing approvals in 2019.

We are currently planning a randomized, controlled global clinical trial for rivo-cel in adult patients with intermediate and high-risk acute myeloid leukemia (AML) or myelodysplastic syndromes (MDS), as well as pediatric patients 12 years and older (US portion only). The trial will compare the primary endpoint of overall survival in patients receiving a haplo-transplant with rivo-cel versus the standard post-Cytosan haplo-transplant regimen. We submitted and reviewed the protocol with the FDA during a Type C meeting and expect to initiate a trial by the end of 2018.

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 1/2 clinical trial in patients with pancreatic, gastric, or prostate cancers expressing PSCA is ongoing and we expect to report initial data from this clinical trial in the fourth quarter of 2018.

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. The ongoing Phase 1/2 clinical trial for BPX-701 is in adult patients with relapsed AML/MDS, or uveal melanoma. Recruitment in



this clinical trial has been slower than projected and we are working to address this by adding clinical trial sites. We expect to report initial data from this clinical trial in 2019.

BPX-602 and BPX-603 are dual-switch GoCAR-T product candidates containing both the iMC activation and CaspaCIDE safety switches. Both candidates have been nominated for advancement to IND applications. We expect to file INDs and initiate Phase 1 clinical trials for both programs in 2019.

We have developed efficient and scalable processes to manufacture genetically modified T cells of high quality, which are currently being used to produce rivo-cel, BPX-601 and BPX-701 for our clinical trials. We are leveraging this know how in combination with our proprietary cellular control technologies, resources, capabilities and expertise for the manufacture of CAR T and TCR product candidates to create and develop first and best-in-class product candidates.

We have established in-house cell manufacturing and vector production capabilities at our headquarters facility in Houston, Texas. We completed the facility build-out in early 2018, and we expect that our facilities will meet our U.S. clinical trial and early commercialization requirements. For the European market, we plan to continue working with established contract manufacturers, with our U.S. manufacturing facility as a potential backup supply source.

#### Recent Developments

On July 13, 2018, we entered into a separation agreement with Alan Musso, our Chief Financial Officer and Treasurer pursuant to which Mr. Musso resigned effective August 31, 2018. We have initiated a search for his replacement and Mr. Musso has agreed to consult with us to provide advice regarding the transition of responsibilities.

In connection with Mr. Musso's resignation, Rosemary Williams, the Company's Vice President of Finance and Controller, will serve as Interim Principal Accounting Officer, and Richard Fair, the Company's Chief Executive Officer will take on the additional role as the Interim Principal Financial Officer, in each case, effective as of August 16, 2018.

On August 6, 2018, we appointed Thierry Darcis, M.D., M.B.A. as General Manager of Europe. Dr. Darcis brings over 20 years of pharmaceutical industry experience spanning global marketing, product development and operations management. Dr. Darcis served as the Executive Vice President and General Manager, Europe of Zogenix, Inc., a clinical-stage company developing treatments for rare central nervous system (CNS) disorders. At Zogenix, he was responsible for establishing the company's pre-commercial presence in Europe and heading the commercial operations for its lead product candidate. Previously, Dr. Darcis served as Vice President and General Manager of the EMEA Region for NPS Pharmaceuticals, Inc., where he was instrumental in the launch of its lead product in various markets, resulting in the successful acquisition of the company by Shire in 2015. Prior to that, he served as Vice President and General Manager of ViroPharma, as well as Director of several ViroPharma subsidiaries.

On October 5, 2018, we entered into an Open Market Sale Agreement with Jefferies LLC, as sales agent, pursuant to which we may offer and sell, from time to time, through Jefferies, shares of the Company's common stock having an aggregate offering price of up to \$60.0 million. The shares will be offered and sold pursuant to the Company's shelf registration statement on Form S-3.

#### 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. We have received funds from the Cancer 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.

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 606. See the discussion of “Collaboration Agreements” contained within Note 3 to the unaudited condensed consolidated financial statements contained herein.

### CPRIT Grant

During 2017, we entered into a grant agreement with CPRIT whereby CPRIT awarded approximately \$16.9 million to fund research of a cancer therapy involving rivo-cel. We received CPRIT funds of \$4.2 million up front which was initially recorded as deferred revenue. From the inception of the CPRIT grant to date, we recognized \$0.9 million of deferred revenue for expenses incurred under the CPRIT Grant. For additional information about the CPRIT grant, see Note 9 to the unaudited condensed consolidated financial statements included herein.

### NIH Grant

During 2013, we entered into a grant agreement with the NIH. The grant was a modular five-year grant with funds being awarded each year based on the progress of the program being funded. Grant money was not received until expenses for the program were incurred. We were awarded approximately \$1.4 million through the grant expiration date of 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 contained within Note 3 to the audited financial statements contained within Item 8 of our Annual Report.

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, acceptance by regulatory authorities, 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 rivo-cel, BPX-601 and BPX-701, and advancing additional product candidates into clinical development, manufacturing registrational, clinical trial and preclinical study materials, expanding our research and development, 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.

We anticipate that our general and administrative expenses will increase in the future to support our continued research and development activities, and the potential commercialization of our product candidates. Specifically, for our rivo-cel program, where we are planning to file MAAs for European marketing approvals in 2019, we expect to build out a focused European commercial team and invest in commercial and medical readiness activities which will increase our general and administrative expenses in future periods.

#### Income Taxes

We did not recognize any income tax expense during either of the nine-month periods ended September 30, 2018 or 2017.

## Results of Operations

## Comparison of the Three and Nine Months Ended September 30, 2018 and 2017

The following table sets forth our results of operations for the three and nine month periods ended September 30, 2018 and 2017 (in thousands):

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2018	2017	Change	2018	2017	Change
Total revenues	\$292	\$126	\$166	\$808	\$254	\$554
Operating expenses:						
Research and development	16,413	18,101	(1,688)	51,361	51,355	6
License fees	139	151	(12 )	319	849	(530 )
General and administrative	6,968	4,579	2,389	18,027	15,992	2,035
Total operating expenses	23,520	22,831	689	69,707	68,196	1,511
Loss from operations	(23,228 )	(22,705 )	(523 )	(68,899 )	(67,942 )	(957 )
Other income (expense):						
Interest income	492	284	208	1,196	788	408
Interest expense	(1,065 )	(1,010 )	(55 )	(3,113 )	(2,707 )	(406 )
Total other expense	(573 )	(726 )	153	(1,917 )	(1,919 )	2
Net loss	\$(23,801)	\$(23,431)	\$(370)	\$(70,816)	\$(69,861)	\$(955)

## Grant Revenues

We recognized grant revenue of \$0.3 million and \$0.8 million, respectively in the three and nine month periods ended September 30, 2018, and \$0.1 million in the three and nine months ended September 30, 2017 from the CPRIT grant. We recognized grant revenue of \$0.2 million in the nine-month period ended September 30, 2017 from the NIH grant, which expired on March 31, 2017.

## Research and Development Expenses

The following table presents our research and development expense by project/category for the three and nine months ended September 30, 2018 and 2017 (in thousands):

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2018	2017	Change	2018	2017	Change
Product Candidates						
Rivo-cel	\$6,049	\$9,559	\$(3,510)	\$21,404	\$27,305	\$(5,901)
BPX-601	865	454	411	2,019	1,571	448
BPX-701	774	323	451	2,017	1,481	536
General	8,725	7,765	960	25,921	20,998	4,923
Total	\$16,413	\$18,101	\$(1,688)	\$51,361	\$51,355	\$6

Research and development expenses in the three months ended September 30, 2018 decreased approximately \$1.7 million, compared with the three months ended September 30, 2017. The decrease is primarily due to decreased research and development activity related to rivo-cel. Fewer patients were enrolled in our rivo-cel clinical trials in 2018 as a result of the FDA clinical hold that took effect in January 2018, and the completion of enrollment in our European BP-004 clinical trial.

Research and development expenses in the nine months ended September 30, 2018 were comparable with the nine month period ended September 30, 2017. Research and development activity related to rivo-cel decreased in the nine-months ended September 30, 2018 compared with the prior year as fewer patients were enrolled in our rivo-cel clinical trials in 2018 as a result of the FDA clinical hold that took effect in January 2018, and the completion of enrollment in our European BP-004 clinical trial. This decrease was offset primarily by the increase in general research and development activity primarily related to increased personnel costs and depreciation costs related to our manufacturing facility which was completed in early 2018.



### License Fees

We incur license fees under the terms of our various license agreements for intellectual property. License fee expense declined \$0.5 million in the nine months ended September 30, 2018 compared with the nine months ended September 30, 2017. License fees in the three and nine months ended September 30, 2017 included milestone payments of \$0.3 million and \$0.5 million, respectively, arising upon the enrollment of the first patient into a clinical trial of BPX-601 and BPX-701. 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 expenses increased \$2.4 million and \$2.0 million, respectively in the three and nine month periods ended September 30, 2018, compared to the same periods in 2017. The increase is primarily due to an increase in personnel costs, arising from severance costs related to former executive officers as well as increased commercialization activities.

### Other Expense

Other expense consists of interest expense partially offset by interest income. Other expense remained flat during the nine month period ending September 30, 2018 compared to the same period in 2017. Other expenses decreased \$0.2 million in the three month period ended September 30, 2018 compared with the same period in 2017 primarily due to increased interest income arising from the investment of proceeds of public offerings of our common stock in March 2017 and April 2018. See Note 8 for additional information about debt obligations. See Note 10 for additional information about the public offerings of our common stock.

### 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, 2018, and December 31, 2017, we had cash, cash equivalents, restricted cash and investment securities of \$118.4 million and \$106.5 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 April 20, 2018, we completed an underwritten public offering of 9,200,000 shares of our common stock at a price of \$7.50 per share, for an aggregate offering size of \$69.0 million, pursuant to a registration statement on Form S-3. The net proceeds to us, after deducting underwriting discounts and commissions and offering expenses, were approximately \$64.7 million.

In August 2018, we filed a registration statement on Form S-3 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 \$150.0 million. The registration statement became effective August 23, 2018.

On October 5, 2018, we entered into an Open Market Sale Agreement with Jefferies LLC, as sales agent, pursuant to which we may offer and sell, from time to time, through Jefferies, shares of the Company’s common stock having an aggregate offering price of up to \$60.0 million. The shares will be offered and sold pursuant to the Company’s shelf registration statement on Form S-3.

#### Cash Flows

The following table sets forth a summary of our cash flows for the nine months ended September 30, 2018 and 2017:

	Nine Months Ended September		
	2018	2017	Change
	(in thousands)		
Net cash used in operating activities	\$(54,381)	\$(60,161)	\$5,780
Net cash provided by investing activities	277	(1,301)	1,578
Net cash provided by financing activities	67,737	76,097	(8,360)
Net change in cash, cash equivalents, and restricted cash	\$13,633	\$14,635	\$(1,002)



### Operating Activities

Net cash used in operating activities for the nine months ended September 30, 2018 was comprised of a net loss of \$70.8 million, which included share-based compensation expense of \$10.9 million, non-cash depreciation expense of \$4.9 million, amortization of deferred financing costs of \$0.7 million and other amortization costs of \$0.1 million. Net cash used in operating activities also included the effect of changes in asset and liability accounts, including an increase of accounts receivable of \$0.2 million, a decrease in prepaids and other assets of \$0.2 million, a decrease in accounts payable of \$1.4 million, an increase in accrued liabilities of \$2.4 million and a decrease of \$0.8 million in deferred revenue.

Net cash used in operating activities for the nine months ended September 30, 2017 was comprised of a net loss of \$69.9 million, which included share-based compensation expense of \$10.2 million, depreciation expense of \$2.5 million and amortization of deferred financing costs of \$0.6 million. Net cash used in operating activities also included an increase in prepaid expenses and other assets of \$0.6 million, and a decrease in accounts payable and other liabilities of \$3.0 million, primarily due to completion of the first phase of construction of our manufacturing facility.

### Investing Activities

Net cash provided by investing activities for the nine months ended September 30, 2018 was \$0.3 million, consisting of the proceeds from sale of investment securities of \$52.2 million, offset by purchases of investment securities totaling \$50.6 million and the purchases of property and equipment of \$1.3 million. Purchases of property and equipment declined \$9.2 million, as we have completed the construction of our manufacturing facility.

Net cash used in investing activities for the nine months ended September 30, 2017 was \$1.3 million, consisting of the proceeds from sale of investment securities of \$45.5 million, offset by the purchase of investment securities of \$36.3 million and the purchase of property and equipment of \$10.5 million.

### Financing Activities

Net cash used in financing activities for the nine months ended September 30, 2018 was \$67.7 million, which was derived from \$64.7 million in net proceeds from our public offering in the second quarter and \$3.1 million proceeds from the exercise of stock options and purchases of our common stock under the employee stock purchase plan.

Net cash provided by financing activities for the nine months ended September 30, 2017 was \$76.1 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 and proceeds from the issuance of our common stock under the employee stock purchase plan of \$1.6 million, partially offset by the payment of debt issuance costs of \$0.1 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.

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 rivo-cel, BPX-601, BPX-701 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;
- successfully negotiating reimbursement for our products from various third-party payors; and

the ability to successfully manufacture patient doses.

24

---



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 our product candidates are in various 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, cash equivalents and investment securities as of September 30, 2018 will enable us to fund our operating expenses and capital expenditure requirements through the end of 2019. 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 rivo-cel, BPX-601 and BPX-701 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, market access 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 rivo-cel 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 September 30, 2018 were as follows:  
(in thousands)

	Less Than 1 Year	1 to 3 Years	3 to 5 Years	More Than 5 Years
License agreements (1)	\$81,719	\$2,683	\$1,314	\$76,470

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

Long-term debt obligations (2)	38,045	—	20,000	18,045	—
Operating lease agreements (3)	10,218	2,072	2,427	2,207	3,512
Research collaborations and sponsored research (4)	1,846	1,846	—	—	—
Manufacturing arrangements (5)	869	869	—	—	—
Manufacturing build-out (6)	445	445	—	—	—
Equipment capital lease agreements (7)	196	68	128	—	—
Total contractual obligations	\$133,338	\$7,983	\$23,869	\$21,504	\$79,982

License agreements - We have entered into several license agreements under which we obtained rights to certain intellectual property. Under the agreements, we could be obligated for payments upon successful completion of (1) clinical and regulatory milestones regarding the products covered by the licenses. The obligations listed in the table above represent estimates of when the milestones will be achieved. The milestones may not be completed when estimated or at all. See Note 12 to the financial statements included in our Annual Report.

(2) Long-term debt obligations - The amounts above consist of obligations under our debt facility. See Note 8 to the financial statements included herein.

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) Research collaborations and sponsored research - We have entered into several research collaborations and sponsored research agreements to undertake research which is of mutual interest to all parties.

Manufacturing arrangements - The amounts above consist of obligations for validation of commercial  
(5) manufacturing processes of rimiducid. The obligations listed in the table above represent estimates of when services will be performed.

(6) 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.

Equipment capital lease agreements - We have entered into a number of office equipment lease agreements with  
(7) various 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 September 30, 2018, we had cash, cash equivalents, restricted cash and investment securities of \$118.4 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 September 30, 2018 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, 2018, 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 (our Principal Executive Officer) and our Principal Accounting Officer, 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 September 30, 2018. 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 September 30, 2018, our Chief Executive Officer and Principal Accounting 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

On February 6, 2018, a purported securities class action complaint captioned Nipun Kakkar v. Bellicum Pharmaceuticals, Inc., Rick Fair and Alan Musso was filed against us, and certain of our officers in the U.S. District Court for the Southern District of Texas, Houston Division. A second substantially similar class action was filed on March 14, 2018 by plaintiff Frances Rudy against the same defendants in the same court. The lawsuits purport to assert class action claims on behalf of purchasers of our securities during the period from May 8, 2017 through January 30, 2018. The complaints allege that the defendants violated the Exchange Act by making materially false and misleading statements concerning our clinical trials being conducted in the U.S. to assess rivo-cel as an adjunct T-cell therapy administered after allogeneic hematopoietic stem cell transplantation. The complaints purport to assert claims for violations of Sections 10(b) and 20(a) of the Exchange Act and Rule 10b-5 promulgated thereunder. The complaints seek, on behalf of the purported class, an unspecified amount of monetary damages, interest, fees and expenses of attorneys and experts, and other relief. On April 9, 2018, the District Court consolidated the two lawsuits under the Kakkar action and motions were filed by putative class members for appointment as lead plaintiff and approval of lead counsel. The District Court has yet to rule on the motions.

On July 19, 2018, a purported shareholder derivative complaint captioned Seung Paik v. Richard A. Fair, et al. was filed against the Company's directors and certain of the Company's officers in the U.S. District Court for the Southern District of Texas, Houston Division. The lawsuit purports to seek damages on behalf of the Company against the individual defendants for breach of fiduciary duty, waste, unjust enrichment and violations of Section 14(a) of the Exchange Act. The complaint alleges that the defendants caused or allowed the Company to disseminate misstatements regarding the clinical trials for rivo-cel and to make false or misleading statements in the proxy materials for the Company's 2017 annual meeting of stockholders. On October 3, 2018, the District Court granted the Company's motion to stay the derivative cause of action until reinstated on motion of the parties.

### Item 1A. Risk Factors

Our business and results of operations are subject to a number of risks and uncertainties. You should carefully consider the following risk factors, as well as the other information in this report, and in our other public filings. The occurrence of any of the following risks could harm our business, financial condition, results of operations and/or growth prospects or cause our actual results to differ materially from those contained in forward-looking statements we have made in this report and those we may make from time to time. You should consider all of the risk factors described when evaluating our business. We have marked with an asterisk (\*) those risk factors that reflect material changes from the risk factors included in our Annual Report on Form 10-K for the year ended December 31, 2017, filed with the Securities and Exchange Commission on March 13, 2018, or our Annual Report.

#### Risks Related to Our Business and Industry

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

We are a clinical stage biopharmaceutical company with a limited operating history. We are not profitable, have no products approved for commercial sale and have incurred significant losses since our inception in 2004. To date, we have financed our operations primarily through equity and debt financings. For the three and nine months ended September 30, 2018, we reported a net loss of \$23.8 million and \$70.8 million, respectively. For the three and nine months ended September 30, 2017, we reported a net loss of \$23.4 million and \$69.9 million, respectively.

As of September 30, 2018, we had an accumulated deficit of \$393.3 million. We expect to continue to incur significant losses for the foreseeable future, and we expect these losses to increase as we continue our research and development of, and seek regulatory approvals for, our product candidates.

In addition, if we obtain regulatory approval of and seek to commercialize any of our product candidates, we will likely incur significant sales, marketing and manufacturing expenses and may continue to incur substantial research and development expenses for additional post-marketing approval development requirements related to such product. We may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business. The size of our future net losses will depend, in part, on the rate of future growth of our

expenses and our ability to generate revenue. Our prior losses and expected future losses have had and will continue to have an adverse effect on our stockholders' equity and working capital.

We will require significant funding to complete the development and commercialization of rivo-cel and any of our other product candidates. If we fail to obtain additional financing, we may have to delay, reduce or eliminate our development programs or commercialization efforts.\*

Our operations have consumed substantial amounts of cash since our inception. We expect to continue to spend substantial amounts to continue the preclinical and clinical development of our product candidates and other research and development programs.

As of September 30, 2018, we had cash and cash equivalents of approximately \$58.7 million and total investments in marketable securities of \$59.7 million. We maintain our cash, cash equivalents, and marketable securities with high quality, accredited financial institutions. These amounts at times may exceed federally insured limits. We believe that cash and cash equivalents and investments in marketable securities of \$118.4 million at September 30, 2018, including the net proceeds of \$64.7 million from our April 2018 public offering of our common stock, will enable us to fund our operating expenses and capital expenditure requirements through the end of 2019. However, changing circumstances may cause us to consume capital significantly faster than we currently anticipate.

We expect to finance future cash needs through public or private equity offerings, debt financings, strategic partnerships and alliances or licensing arrangements. We cannot be certain that additional funding will be available on acceptable terms, or at all. Subject to limited exceptions, our loan agreement with Oxford Finance prohibits us from incurring indebtedness without the prior written consent of Oxford. If we are unable to raise additional capital in sufficient amounts or on terms acceptable to us we will need to significantly delay, scale back or discontinue the development or commercialization of our product candidates. We also could be required to:

- seek collaborators for one or more of our current or future product candidates on terms that are less favorable than might otherwise be available;

- relinquish or license on unfavorable terms our rights to technologies or product candidates that we otherwise would seek to develop or commercialize ourselves; or

- seek a third party to acquire us or our assets.

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

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

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

- completing requisite clinical trials through all phases of clinical development of rivo-cel and our other current product candidates;

- seeking and obtaining marketing approvals for rivo-cel and any other product candidates that successfully complete clinical trials, if any;

- launching and commercializing rivo-cel and other product candidates for which we obtain marketing approval, if any, with a partner or, if launched independently, successfully establishing a sales force, marketing and distribution infrastructure;

- identifying and developing new product candidates;

- progressing our pre-clinical programs into human clinical trials;

- establishing and maintaining supply and manufacturing relationships with third parties;

- developing new molecular switches based on our proprietary CID technology platform;

- maintaining, protecting, expanding and enforcing our intellectual property; and

- attracting, hiring and retaining qualified personnel.





Because of the numerous risks and uncertainties associated with biologic product development, we are unable to predict the likelihood or timing for when we may receive regulatory approval of rivo-cel or any of our other current or future product candidates or when we will be able to achieve or maintain profitability, if ever. If we do not receive regulatory approvals, our business, prospects, financial condition and results of operations will be adversely affected. Even if we obtain the regulatory approvals to market and sell one or more of our product candidates, we may never generate significant revenues from any commercial sales for several reasons, including because the market for our products may be smaller than we anticipate, or products may not be adopted by physicians and payors or because our products may not be as efficacious or safe as other treatment options. If we fail to successfully commercialize one or more products, we may be unable to generate sufficient revenues to sustain and grow our business and our business, prospects, financial condition and results of operations will be adversely affected. In addition, our expenses could increase beyond expectations if we are required by the European Medicines Agency, or EMA, the FDA, or other foreign regulatory agencies, to perform studies and clinical trials in addition to those that we currently anticipate for rivo-cel and our other product candidates, or if there are any delays in our or our partners completing clinical trials or the development of any of our product candidates. Further, if one or more of the product candidates that we independently develop is approved for commercial sale, we expect to incur significant costs associated with commercializing any such product candidates. Finally, even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis.

Our CID technology is novel and largely unproven.

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

CAR T and TCR cell therapies are novel and present significant challenges.\*

CAR T and TCR product candidates represent a relatively new field of cellular immunotherapy. Advancing this novel and personalized therapy creates significant challenges for us, including:

- obtaining regulatory approval, as the EMA, FDA and other regulatory authorities have limited experience with commercial development of T-cell therapies for cancer;
- sourcing clinical and, if approved, commercial supplies for the materials used to manufacture and process our product candidates;
- developing a consistent and reliable process, while limiting contamination risks, for engineering and manufacturing T cells ex vivo and infusing the engineered T cells into the patient;
- educating medical personnel regarding the potential safety benefits, as well as the challenges, of incorporating our product candidates into their treatment regimens;
- establishing sales, marketing, and market access upon obtaining any regulatory approval to gain market acceptance of a novel therapy; and
- the availability of coverage and adequate reimbursement from third-party payors for our novel and personalized therapy.

Our inability to successfully develop CAR T and TCR cell therapies or develop processes related to the manufacture or commercialization of these therapies would adversely affect our business, results of operations and prospects.

The EMA and/or FDA may disagree with our regulatory plans and we may fail to obtain regulatory approval of our product candidates.\*

Our business and future success depends, in part, on our ability to obtain regulatory approval of and then successfully commercialize rivo-cel and our other clinical product candidates. All of our product candidates, including rivo-cel, will require additional clinical and non-clinical development, regulatory review and approval in multiple jurisdictions, substantial investment, and access to sufficient commercial manufacturing capacity and significant marketing efforts before we can expect to generate any revenue from product sales.



rivo-cel and our other product candidates could fail to receive regulatory approval for many reasons, including the following:

- the EMA, FDA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials;
- we may be unable to demonstrate to the satisfaction of the EMA, FDA or comparable foreign regulatory authorities that our product candidates have the necessary safety, purity, and potency for any of their proposed indications;
- the results of clinical trials may not meet the level of statistical significance required by the EMA, FDA or comparable foreign regulatory authorities for approval;
- we may be unable to demonstrate that our product candidates' clinical and other benefits outweigh their safety risks;
- we may encounter serious and unexpected adverse events during clinical trials that render our products unsafe for use in humans;
- the EMA, FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from preclinical studies or clinical trials;
- the data collected from clinical trials of our product candidates may not be sufficient to the satisfaction of the EMA, FDA or comparable foreign regulatory authorities to support the submission of an MAA, BLA or other comparable submission in foreign jurisdictions or to obtain regulatory approval in Europe, the U.S. or elsewhere;
- the EMA, FDA or comparable foreign regulatory authorities may fail to approve our manufacturing processes and/or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; and
- the approval policies or regulations of the EMA, FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

We plan to initially seek approval for rivo-cel and rimiducid from the EMA for the treatment of pediatric patients undergoing haploidentical (partial match) hematopoietic stem cell transplants, or HSCT, and to submit the MAAs, for this indication in 2019. While we expect that the European arm of our BP-004 trial could serve as the registrational trial for these MAAs, this clinical trial was not originally designed for that purpose. We cannot be certain that our preclinical and clinical trial package for the MAAs will be sufficient for approval of rivo-cel for multiple reasons including issues related to trial conduct and analysis; limitations of data available from pre-clinical and Phase ½ studies; or issues related to CMC efforts to date. We have sought to avoid or remediate potential issues but we cannot be sure that such efforts will be effective or sufficient. Further, we cannot assure you that the EMA or any other regulatory agency will agree that rivo-cel provides a clinically meaningful and differentiated therapeutic benefit or that the side effects experienced in our clinical trials yield an acceptable benefit/risk ratio in the opinion of the EMA or other regulatory agencies. If the MAAs for rivo-cel are deficient, we will incur additional expenses to address the deficiencies, which may require additional clinical trials, and the commercialization of rivo-cel will be delayed. This would adversely affect our business, results of operations and prospects.

We, or our institutional collaborators, are conducting and planning additional clinical trials of rivo-cel designed to support FDA approval of our therapy in other indications. In each case, we plan to conduct one clinical trial to support registration in that indication. However, the general approach for FDA approval of a new biologic or drug is to require dispositive data from two adequate and well-controlled, Phase 3 clinical trials of the relevant biologic or drug in the relevant patient population. Phase 3 clinical trials typically involve hundreds of patients, have significant costs and take years to complete. We believe that a single Phase 3 clinical trial strategy is warranted given the limited alternatives for patients for which rivo-cel therapy is potentially beneficial, but the FDA may ultimately require more than one Phase 3 clinical trial and may limit clinical trial designs allowed to serve as a registration trial.

In addition, because rivo-cel is our most advanced product candidate, and because many of our other product candidates are based on similar technology, if rivo-cel encounters safety or efficacy problems, developmental delays, regulatory issues or other problems, our development plans and business for our other product candidates would be significantly harmed.

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

Clinical testing is expensive, takes many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical trial process and our product candidates are subject to the risks of failure inherent in biologic drug development. Success in early clinical trials does not mean that later clinical trials will be successful because product candidates in later-stage clinical trials may fail to demonstrate sufficient safety or efficacy despite having progressed through initial clinical testing, even at statistically significant levels. We will be required to demonstrate through clinical trials that our product candidates are safe and effective for use in the target indication before we can obtain regulatory approvals for commercial sale. Companies frequently suffer significant setbacks in late-stage clinical trials, even after earlier clinical trials have shown promising results and most product candidates that commence clinical trials are never approved as products. We expect there may be greater variability in results for cellular immunotherapy products processed and administered on a patient-by-patient basis like all our CID technology-based development and product candidates than for “off-the-shelf” products, like many drugs. If any of our product candidates fail to demonstrate sufficient safety or efficacy, we would experience potentially significant delays in, or be required to abandon our development of the product candidate, which would have a material and adverse impact on our business, prospects, financial condition and results of operations. Many of our current product candidates are in early stage clinical trials, and we may experience unfavorable results in the future.\*

We have begun enrolling patients in Phase 1 clinical trials of BPX-601 for the treatment of pancreatic, gastric, and prostate cancers and BPX-701 for the treatment of refractory or relapsed AML/MDS and uveal melanoma. We have not initiated clinical trials for any additional preclinical product candidates and we may not be able to commence clinical trials on the time frames we expect. As these product candidates are in early stages of development, we face significant uncertainty regarding how effective and safe they will be in human patients and the results from preclinical studies, such as in vitro and in vivo studies, of BPX-601 and BPX-701 and our other preclinical programs may not be indicative of the results of clinical trials of these product candidates. Preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval for their products.

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

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

We believe that our CID platform, which serves as the foundation of our CaspaCIDE and GoCAR-T technologies, can be further leveraged to discover other novel technologies, therapeutic applications and market opportunities. For example, we are developing new molecular switches and dual-switch systems to provide greater control over cellular immunotherapy. We are at an early stage of development and our platform has not yet, and may never lead to, approved or marketable products. Even if we are successful in continuing to build our pipeline, the potential product candidates that we identify may not be suitable for clinical development, including for reasons related to their harmful side effects, limited efficacy or other characteristics that indicate that they are unlikely to be products that will receive marketing approval and achieve market acceptance. If we do not successfully develop and commercialize product candidates based upon our technological approach, we may not be able to obtain product or partnership revenues in future periods, which would adversely affect our business, prospects, financial condition and results of operations. We rely and will continue to rely on third parties to conduct our clinical trials. If these third parties do not successfully carry out their contractual duties or meet expected deadlines, we may not be able to obtain regulatory approval of or

commercialize our product candidates.

We depend and will continue to depend upon independent investigators and collaborators, such as universities, medical institutions, and strategic partners to conduct our preclinical and clinical trials under agreements with us. Negotiations of

32

---

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

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

Switching or adding third parties to conduct our clinical trials involves substantial cost and requires extensive management time and focus. In addition, there is a natural transition period when a new third party commences work. As a result, delays occur, which can materially impact our ability to meet our desired clinical development timelines. Also, we are conducting multiple clinical trials in Europe and may plan additional testing of our technology and product candidates in other foreign jurisdictions. We currently have limited staffing and capabilities in foreign countries and may not be able to effectively resolve potential disputes with our independent investigators and collaborators.

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

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

- the patient eligibility criteria defined in the protocol;
- the size of the patient population required for analysis of the trial's primary endpoints;
- the proximity of patients to study sites;
- the design of the clinical trial;
- our ability to recruit clinical trial investigators with the appropriate competencies and experience;
- our ability to obtain and maintain patient consents;
- the risk that patients enrolled in clinical trials will drop out of the clinical trials before completion; and
- competing clinical trials and approved therapies available for patients.





In particular, some of our clinical trials will look to enroll patients with characteristics which are found in a very small population, for example, patients with rare cancers with specific attributes that are targeted with our product candidates. Our clinical trials will compete with other companies' clinical trials for product candidates that are in the same therapeutic areas as our product candidates, and this competition will reduce the number and types of patients available to us, because some patients who might have opted to enroll in our clinical trials may instead opt to enroll in a trial being conducted by one of our competitors. Since the number of qualified clinical investigators is limited, we expect to conduct some of our clinical trials at the same clinical trial sites that some of our competitors use, which will reduce the number of patients who are available for our clinical trials in these clinical trial sites. Moreover, because our product candidates represent a departure from more commonly used methods for cancer treatment, potential patients and their doctors may be inclined to use conventional therapies, such as chemotherapy and antibody therapy, rather than enroll patients in any of our future clinical trials. Patients may also be unwilling to participate in our clinical trials because of negative publicity from adverse events in the biotechnology or gene therapy industries. Delays in patient enrollment may result in increased costs or may affect the timing or outcome of the planned clinical trials, which could prevent completion of these clinical trials and adversely affect our ability to advance the development of our product candidates.

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

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

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

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

Undesirable side effects observed in our clinical trials, whether or not they are caused by our product candidates, could result in the delay, suspension or termination of clinical trials by us, the FDA or other regulatory authorities for a number of reasons. In addition, because the patients in our clinical trials are suffering from life-threatening diseases, are often suffering from multiple complicating conditions and, in the case of transplant patients, are in a position of extreme immune deficiency at the time that they receive our therapy, it may be difficult to accurately assess the relationship between our product candidates and adverse events experienced by very ill patients. For example, in January 2018, we announced that we had received notice from the FDA that a clinical hold had been placed on our U.S. clinical trials of rivo-cel following three cases of encephalopathy deemed as possibly related to rivo-cel. In April 2018, we announced that the FDA had lifted the clinical hold following consultation between us and the FDA and agreement on amendments to the study protocols, including guidance on monitoring and management of certain neurologic adverse events. The FDA or foreign regulatory authorities, including in Europe, could in the future take similar actions, which would harm our business. If we elect or are required to delay, suspend or terminate any clinical

trial of any product candidates that we develop, the commercial prospects of such product candidates will be harmed and our ability to generate product revenues from any of these product candidates will be delayed or eliminated. Serious adverse events observed in clinical trials could hinder or prevent market acceptance of the product candidate at issue. Any of these occurrences may harm our business, prospects, financial condition and results of operations significantly.

34

---

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

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

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

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

Specifically, genetically engineering T cells faces significant competition in both the CAR and TCR technology space from multiple companies, including Adaptimmune, bluebird bio, Inc., Celgene Corporation, Collectis SA, Gilead, GlaxoSmithKline plc, Intrexon Corporation, Juno Therapeutics, Inc., Kite Pharma, Inc., Novartis AG, Pfizer Inc. and Ziopharm Oncology. Our most advanced product candidate, rivo-cel, is an adjunct therapy for HSCT with alternative donors that potentially provides improved outcomes by accelerating the reconstitution of the host immune system and addressing the safety risks of GvHD. Other companies are developing product candidates to improve the outcome of HSCT, including Kiadis Pharma Netherlands B.V., MolMed S.p.A., and Dompe Farmaceutici S.p.A.. Even if we obtain regulatory approval of our product candidates, the availability and price of our competitors' products could limit the demand and the price we are able to charge for our product candidates. We may not be able to implement our business plan if the acceptance of our product candidates is inhibited by price competition or the reluctance of physicians to switch from existing methods of treatment to our product candidates, or if physicians switch to other new drug or biologic products or choose to reserve our product candidates for use in limited circumstances. For additional information regarding our competition, see "Item 1. Business—Competition" under Part I of our Annual Report. Rivo-cel and rimiducid have received orphan drug designation, but we may be unable to maintain or receive the benefits associated with orphan drug status, including market exclusivity.

The FDA or EMA grant orphan designation to a drug or biologic intended to treat a rare disease or condition or for which there is no reasonable expectation that the cost of developing and making available in that jurisdiction a drug or biologic for a disease or condition will be recovered from sales in that jurisdiction for that drug or biologic. If a product that has orphan drug designation subsequently receives the first FDA or EMA approval for the disease for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA or EMA may not approve any other applications, including a full authorization to market the same biologic for the same

indication for seven years in the U.S. and for 10 years in Europe, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity.

The EMA has granted orphan drug designations to rivo-cel for treatment in HSCT, and for activator agent rimiducid for the treatment of GvHD. Additionally, rivo-cel and rimiducid have received orphan drug designation from the FDA, as a combination replacement T-cell therapy for the treatment of immunodeficiency and GvHD after allogeneic HSCT. However, in

35

---

each case, exclusive marketing rights may be limited if we seek approval for an indication broader than the orphan designated indication and may be lost if the EMA or FDA, as applicable, later determines that the request for designation was materially defective or if we are unable to assure the availability of sufficient quantities of the product to meet the needs of patients with the rare disease or condition. Although the respective designations may provide seven years of market exclusivity in the U.S. and 10 years of market exclusivity in Europe, the designations are subject to certain limited exceptions. Therefore, even though we have obtained orphan drug designation for certain indications, we may be unable to obtain orphan drug designation for our future product candidates and we may not be the first to obtain marketing approval for any particular orphan indication.

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

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

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

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

As our development and commercialization plans and strategies develop, including the preparations for a potential launch of rivo-cel in Europe, we expect to need additional managerial, medical, operational, sales, marketing, market access financial and other personnel. Future growth imposes significant added responsibilities on members of management, including:

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

There are a small number of individuals with experience in cell therapy and the competition for these individuals is high. Our future financial performance and our ability to commercialize our product candidates will depend, in part, on our ability to effectively manage any future growth, and our management may also have to divert a disproportionate amount of its attention away from day-to-day activities to devote a substantial amount of time to managing these growth activities. We currently rely, and for the foreseeable future will continue to rely, in substantial part on certain independent organizations, advisors and consultants to provide certain services, including substantially all aspects of regulatory approval, clinical management, and manufacturing. The services of independent organizations, advisors and consultants may not continue to be available to us on a timely basis when needed, and we may not be able to find qualified replacements. In addition, if we are unable to effectively manage our outsourced activities or if the quality or accuracy of the services provided by consultants is compromised for any reason, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval of our product candidates or otherwise advance our business. We may not be able to manage our existing consultants or find other competent outside contractors and consultants on economically reasonable terms, or at all.

If we are not able to effectively expand our organization by hiring new employees and expanding our groups of consultants and contractors, we may not be able to successfully implement the tasks necessary to further develop and

commercialize our product candidates, and, accordingly, may not achieve our research, development and commercialization goals.

The terms of our debt facility place restrictions on our operating and financial flexibility, and failure to comply with covenants or to satisfy certain conditions of the agreement governing the debt facility may result in acceleration of our

36

---

repayment obligations and foreclosure on our pledged assets, which could significantly harm our liquidity, financial condition, operating results, business and prospects and cause the price of our common stock to decline.

In December 2017, we entered into a loan and security agreement with Oxford Finance LLC, or Oxford, that is secured by a lien covering substantially all of our assets, excluding intellectual property, but including proceeds from the sale, license, or disposition of our intellectual property, under which we have borrowed \$35.0 million. The loan and security agreement governing the debt facility requires us to comply with a number of covenants (affirmative and negative), including restrictive covenants that limit our ability to: incur additional indebtedness; encumber the collateral securing the loan; acquire, own or make investments; repurchase or redeem any class of stock or other equity interest; declare or pay any cash dividend or make a cash distribution on any class of stock or other equity interest; transfer a material portion of our assets; acquire other businesses; and merge or consolidate with or into any other organization or otherwise suffer a change in control, in each case subject to exceptions. Our intellectual property also is subject to customary negative covenants. In addition, subject to limited exceptions, Oxford could declare an event of default upon the occurrence of any event that it interprets as having a material adverse effect upon our business, operations, properties, assets, or financial condition or upon our ability to perform or pay the secured obligations under the loan and security agreement or upon the collateral or Oxford's liens on the collateral under the agreement, thereby requiring us to repay the loan immediately, together with a prepayment charge of up to 3% of the then outstanding principal balance and an end-of-term charge. Although, in and of itself, the occurrence of adverse results or delays in any clinical study or the denial, delay or limitation of approval of or taking of any other regulatory action by the FDA or another governmental entity will not constitute a material adverse effect under our loan and security agreement with Oxford, Oxford may determine that such an event together with contemporaneous events or circumstances constitutes a material adverse effect upon our business, operations, properties, assets, or financial condition or upon our ability to perform or pay the secured obligations under the loan and security agreement. If we default under the facility, Oxford may accelerate all of our repayment obligations and, if we are unable to access funds to meet those obligations or to renegotiate our agreement, Oxford could take control of our pledged assets and we could immediately cease operations. If we were to renegotiate our agreement under such circumstances, the terms may be significantly less favorable to us. If we were liquidated, Oxford's right to repayment would be senior to the rights of our stockholders to receive any proceeds from the liquidation. Any declaration by Oxford of an event of default could significantly harm our liquidity, financial condition, operating results, business, and prospects and cause the price of our common stock to decline.

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

We need to oversee manufacturing of a complex supply chain of cellular therapy product candidates, viral vectors and small molecule drugs. We expect to rely on third parties to manufacture a substantial portion of our clinical cell therapy product candidates, viral vectors and small molecule supplies in Europe.

Because of the complex nature of our products, we need to oversee the manufacture of multiple components that require a diverse knowledge base and appropriate manufacturing personnel. The supply chain for these components is separate and distinct, and no single manufacturer can supply more than one component of each of our products.

Additionally, it is likely that the cell therapy products will need to be made within an appropriate geographic location for the area in which the products will be utilized, so one cell therapy manufacturing facility may not be able to supply diverse geographic areas. Any lack of capabilities to store, freeze, thaw and infuse our cell therapies would adversely affect our business and prospects.

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

We may be unable to identify manufacturers on acceptable terms or at all because the number of potential manufacturers is limited, and any replacement contractor must be approved by the EMA. This approval would require new testing and compliance inspections. In addition, a new manufacturer would have to be educated in, or develop substantially equivalent processes for, production of our products after receipt of regulatory approval, if any. Our third-party manufacturers might be unable to timely formulate and manufacture our product or produce the quantity and quality required to meet our clinical and commercial needs, if any.



Our future contract manufacturers may not perform as agreed or may not remain in the contract manufacturing business for the time required to supply our clinical trials or to successfully produce, store and distribute our products. Manufacturers are subject to ongoing periodic unannounced inspection by regulatory agencies to ensure strict compliance with cGMP and other government regulations and standards. We do not have control over third-party manufacturers' compliance with these regulations and standards.

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

Our third-party manufacturers could breach or terminate their agreement with us.

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

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

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

We have begun limited in-house manufacturing at our own manufacturing facility for supply of U.S. clinical product candidate requirements, and anticipated using this facility to meet US commercial cell therapy product requirements. This will require significant resources and expertise and we may fail to successfully complete or grow our manufacturing capabilities as planned, which could adversely affect our clinical trials and the commercial viability of our product candidates.\*

We have completed the buildout of manufacturing space at our leased headquarters in Houston, Texas and have begun in-house clinical manufacturing. We also rely on outside vendors to manufacture clinical supplies and process intermediates to support our clinical trials. Internal manufacturing for clinical trial and future commercial use will rely upon finding personnel with appropriate background and training to staff and operate the facility on a daily basis. Should we be unable to find these individuals, we may need to rely on external contractors longer than anticipated, and train additional personnel to fill the needed roles. There are a small number of individuals with experience in cell therapy and the competition for these individuals is high.

Specifically, the operation of a cell-therapy manufacturing facility is a complex endeavor requiring knowledgeable individuals who have successful previous experience in cleanroom environments. Cell therapy facilities, like other biological agent manufacturing facilities, require appropriate commissioning and validation activities to demonstrate that they operate as designed. Additionally, each manufacturing process must be proven through the performance of process validation runs to guarantee that the facility, personnel, equipment, and process work as designed. While we have developed our own manufacturing processes using an in-house team, there is timing risk associated with increased in-house product manufacture.

The manufacture of our product candidates is complex and requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. Manufacturers of cell therapy products often encounter difficulties in production, particularly in scaling out and validating initial production and ensuring the absence of contamination. These include difficulties with production costs and yields, quality control, including stability of the product, quality assurance testing, operator error, shortages of qualified personnel, as well as compliance with strictly enforced federal, state and foreign regulations. Furthermore, if contaminants are discovered in our supply of product candidates or in our manufacturing facilities, the manufacturing facilities may need to be closed for an extended period to investigate and remedy the contamination. It is possible that stability or other issues relating to the manufacture of our product candidates could occur in the future.

Our product candidates currently are and will continue to be manufactured on a patient-by-patient basis. We have not yet manufactured our clinical trial product candidates on a large scale, nor on a commercial scale, and may not be able to achieve

38

---

large scale clinical trial or commercial manufacturing and processing on our own to satisfy expected clinical trial or commercial demands for any of our product candidates. While we believe that our current manufacturing and processing approaches are appropriate to support our clinical product development, we have limited experience in managing the T cell engineering process, and our processes may be more difficult or more expensive than anticipated. The manufacturing processes employed by us may not result in product candidates that will be safe and effective. Our manufacturing operations will be subject to review and oversight by the FDA upon commencement of the manufacturing of our product candidates for our planned Phase 3 clinical trials. We will have to complete facility validation, and must obtain approval from the FDA prior to licensure to manufacture our product candidates for these trials. Even if approved, we will continue to be subject to ongoing periodic unannounced inspection by the FDA, the Drug Enforcement Administration and corresponding state agencies to ensure strict compliance with current good manufacturing practices and other government regulations. Our license to manufacture product candidates will be subject to continued regulatory review.

We do not yet have sufficient information to reliably estimate the cost of commercial manufacturing and processing of our product candidates. The actual cost to manufacture and process our product candidates could materially and adversely affect the commercial viability of our product candidates. As a result, we may never be able to develop a commercially viable product.

We also may fail to manage the logistics of collecting and shipping patient material to our manufacturing site and shipping the product candidate back to the patient. Logistical and shipment delays and problems, whether or not caused by us or our vendors, could prevent or delay the delivery of product candidates to patients.

In addition, it is possible that we could experience manufacturing difficulties in the future due to resource constraints or because of labor disputes. If we were to encounter any of these difficulties, our ability to provide our product candidates to patients could be materially adversely affected.

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

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

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

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

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

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



We may not be able to develop in-house market access, marketing, sales and distribution capabilities or establish or maintain relationships with third-party collaborators to commercialize any product in Europe or the U.S.

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

We plan to seek regulatory approval of our product candidates, including rivo-cel, outside of the U.S. and, accordingly, we will be subject to additional risks related to operating in foreign countries if we obtain the necessary approvals, including:

- differing regulatory requirements in foreign countries;
- unexpected changes in tariffs, trade barriers, price and exchange controls and other regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign taxes, including withholding of payroll taxes;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country;
- difficulties staffing and managing foreign operations;
- workforce uncertainty in countries where labor unrest is more common than in the U.S.;
- potential liability under the FCPA or comparable foreign regulations;
- challenges enforcing our contractual and intellectual property rights, especially in those foreign countries that do not respect and protect intellectual property rights to the same extent as the U.S.;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad;
- differing payor reimbursement regimes, governmental payors or patient self-pay systems and price controls; and
- business interruptions resulting from geo-political actions, including war and terrorism.

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

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

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

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

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



collaborators, advisors, employees and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, such as trade secrets. Despite these contractual provisions, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's discovery of our trade secrets or other unauthorized use or disclosure would impair our competitive position and may have a material adverse effect on our business. We may use our financial and human resources to pursue a particular research program or product candidate and fail to capitalize on programs or product candidates that may be more profitable or for which there is a greater likelihood of success.

Because we have limited resources, we may forego or delay pursuit of opportunities with certain programs or product candidates or for indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs for product candidates may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through strategic collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate, or we may allocate internal resources to a product candidate in a therapeutic area in which it would have been more advantageous to enter into a partnering arrangement.

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

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

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

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

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

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

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

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

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

the federal Anti-Kickback Statute, which prohibits, among other things, knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe, or rebate), directly or indirectly, overtly or covertly, in cash or in kind, to induce, or in return for, either the referral of an individual for, or the purchase, lease,



order or recommendation of any good, facility, item or service for which payment may be made, in whole or in part, under a federal healthcare program, such as the Medicare and Medicaid programs;  
federal civil and criminal false claims laws and civil monetary penalties law, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid, or other third-party payors that are false or fraudulent or knowingly making a false statement to improperly avoid, decrease or conceal an obligation to pay money to the federal government;

HIPAA, which created new federal criminal statutes that prohibit knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or obtain, by means of false or fraudulent pretenses, representations, or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, regardless of the payor (e.g., public or private) and knowingly and willfully falsifying, concealing or covering up by any trick or device a material fact or making any materially false statements in connection with the delivery of, or payment for, healthcare benefits, items or services relating to healthcare matters; HIPAA, as amended HITECH, and their respective implementing regulations, which impose requirements on certain covered healthcare providers, health plans, and healthcare clearinghouses as well as their respective business associates that perform services for them that involve the use, or disclosure of, individually identifiable health information, relating to the privacy, security and transmission of individually identifiable health information without appropriate authorization;

the federal Physician Payment Sunshine Act, and its implementing regulations, which requires certain manufacturers of drugs, devices, biologicals and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) to report annually to the HHS, information related to payments or other transfers of value made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, as well as require certain manufacturers and group purchasing organizations to report annually ownership and investment interests held by physicians and their immediate family members; federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers; and

foreign laws that govern the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by or are in conflict with HIPAA, including the European Union General Data Protection Regulation (GDPR), which became effective on May 25, 2018, and which imposes privacy and security obligations on any entity that collects and/or processes health data from individuals located in the European Union. Under the GDPR, fines of up to 20 million euros or up to 4% of the annual global turnover of the infringer, whichever is greater, could be imposed for significant non-compliance. As well as complicating our compliance efforts, non-compliance with these laws could result in penalties or significant legal liability.

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

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

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

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

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

Our inability to obtain sufficient product liability insurance at an acceptable cost to protect against potential product liability claims could prevent or inhibit the commercialization of any products we develop, alone or with corporate collaborators. We currently carry \$10.0 million of product liability insurance covering our clinical trials, with other coverage limits as appropriate for certain foreign jurisdictions. Although we maintain such insurance, our insurance policies may have various exclusions, and we may be subject to a product liability claim for which we have no coverage. We may have to pay any amounts awarded by a court or negotiated in a settlement that exceed our coverage limitations or that are not covered by our insurance, and we may not have, or be able to obtain, sufficient capital to pay such amounts. Even if our agreements with any future corporate collaborators entitle us to indemnification against losses, such indemnification may not be available or adequate should any claim arise.

The recently passed comprehensive tax reform bill could adversely affect our business and financial condition. On December 22, 2017, the president of the United States signed into law new legislation that significantly revises the Internal Revenue Code of 1986, as amended. The newly enacted federal income tax law, among other things, contains significant changes to corporate taxation, including reduction of the corporate tax rate from a top marginal rate of 35% to a flat rate of 21%, limitation of the tax deduction for interest expense to 30% of adjusted earnings (except for certain small businesses), limitation of the deduction for net operating losses to 80% of current year taxable income and elimination of net operating loss carrybacks, one time taxation of offshore earnings at reduced rates regardless of whether they are repatriated, elimination of U.S. tax on foreign earnings (subject to certain important exceptions), immediate deductions for certain new investments instead of deductions for depreciation expense over time, and modifying or repealing many business deductions and credits. Notwithstanding the reduction in the corporate income tax rate, the overall impact of the new federal tax law is uncertain and our business and financial condition could be adversely affected. In addition, it is uncertain if and to what extent various states will conform to the newly enacted federal tax law.

Our ability to utilize our net operating loss carryforwards and certain other tax attributes may be limited.\* Under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, if a corporation undergoes an "ownership change," generally defined as a greater than 50% change (by value) in its equity ownership over a three-year period, the corporation's ability to use its pre-change net operating loss carryforwards and other pre-change tax attributes to offset its post-change income may be limited. As a result of our initial IPO in December 2014 and our

private placements and other

44

---

transactions that have occurred over the past three years, we may have experienced an “ownership change.” We may also experience ownership changes in the future as a result of subsequent shifts in our stock ownership. Our NOL carryforwards will expire beginning in 2025, if not utilized. As a result of the 2017 tax reform act, new NOLs generated in 2018 and thereafter will no longer be subject to expiration, but will be subject to an 80% limitation.

#### Risks Related to Government Regulation

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

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

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

- the availability of financial resources to commence and complete our planned clinical trials;
- reaching agreement on acceptable terms with prospective clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different clinical trial sites;
- recruiting suitable patients to participate in a clinical trial;
- having patients complete a clinical trial or return for post-treatment follow-up;
- clinical trial sites deviating from clinical trial protocol, failing to follow GCPs, or dropping out of a clinical trial;
- adding new clinical trial sites; or
- manufacturing sufficient quantities of qualified materials under cGMPs and applying them on a subject by subject basis for use in clinical trials.

Gene therapy clinical trials conducted at institutions that receive funding for recombinant DNA research from the NIH, are also subject to review by its Recombinant DNA Advisor Committee, or RAC. Although the FDA decides whether individual gene therapy protocols may proceed, the RAC review process can delay the initiation of a clinical trial, even if the FDA has reviewed the trial design and details and approved its initiation. Conversely, the FDA can put an IND on clinical hold even if the RAC has provided a favorable review of the drug. For example, in January 2018 we announced that we had received notice from the FDA that a clinical hold had been placed on our U.S. clinical trials of rivo-cel following three cases of encephalopathy deemed as possibly related to rivo-cel. In April 2018, we announced that the FDA had lifted the clinical hold following consultation between us and the FDA and agreement on amendments to the study protocols, including guidance on monitoring and management of certain neurologic adverse events.

Also, before a clinical trial can begin at an NIH-funded institution, that institution’s independent institutional review board, or IRB, and its Institutional Biosafety Committee must review the proposed clinical trial to assess the safety of the trial. In addition, adverse developments in clinical trials of gene therapy products conducted by others may cause the FDA or other regulatory bodies to change the requirements for approval of any of our product candidates.

We could also encounter delays if physicians encounter unresolved ethical issues associated with enrolling patients in clinical trials of our product candidates in lieu of prescribing existing treatments that have established safety and efficacy profiles. Further, a clinical trial may be suspended or terminated by us, the IRBs for the institutions in which such clinical trials are being conducted, the Data Monitoring Committee for such clinical trial, or by the FDA or other regulatory authorities due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or clinical trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse

side effects, failure to demonstrate a benefit from using a product candidate, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial. If we experience termination of, or delays in the completion of, any clinical trial of our product candidates, the

45

---

commercial prospects for our product candidates will be harmed, and our ability to generate product revenue will be delayed. In addition, any delays in completing our clinical trials will increase our costs, slow down our product development and approval process and jeopardize our ability to commence product sales and generate revenue. Many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may ultimately lead to the denial of regulatory approval of our product candidates.

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

Obtaining and maintaining regulatory approval of our product candidates in one jurisdiction does not guarantee that we will be able to obtain or maintain regulatory approval in any other jurisdiction, while a failure or delay in obtaining regulatory approval in one jurisdiction may have a negative effect on the regulatory approval process in others.

Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and greater than, those in the EU or U.S., including additional preclinical studies or clinical trials.

Studies and clinical trials conducted in one jurisdiction may not be accepted by regulatory authorities in other jurisdictions.

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

There are also foreign regulations governing the privacy and security of health information and the use of personal information to sell or market products, including the GDPR, which became effective on May 25, 2018, and which imposes privacy and security obligations on any entity that collects and/or processes health data from individuals located in the European Union and/or sells or markets products in the European Union. Under the GDPR, fines of up to 20 million euros or up to 4% of the annual global turnover of the infringer, whichever is greater, could be imposed for significant non-compliance.

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

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

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

- fines, warning letters or holds on clinical trials;

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

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

product seizure or detention, or refusal to permit the import or export of our product candidates; and injunctions or the imposition of civil or criminal penalties.



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

Foreign legislative changes may also affect our ability to commercialize our product candidates. Effective as of May 25, 2018, the GDPR imposes privacy and security obligations on any entity that collects and/or processes personal information from individuals located in the European Union. Under the GDPR, fines of up to 20 million euros or up to 4% of the annual global turnover of the infringer, whichever is greater, could be imposed for significant non-compliance.

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

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

- the clinical indications for which our product candidates are approved;
- physicians, hospitals, cancer treatment centers and patients considering our product candidates as a safe and effective treatment;
- the potential and perceived advantages of our product candidates over alternative treatments;
- the prevalence and severity of any side effects;
- product labeling or product insert requirements of the EMA, FDA or other regulatory authorities;
- limitations or warnings contained in the labeling approved by the EMA, FDA or other regulatory authorities;
- the extent and quality of the clinical evidence supporting the efficacy and safety of our product candidates;
- the timing of market introduction of our product candidates as well as competitive products;
- the cost of treatment in relation to alternative treatments;
- the availability of adequate reimbursement and pricing by third-party payors and government authorities;
- the willingness and ability of patients to pay out-of-pocket in the absence of coverage by third-party payors, including government authorities;
- relative convenience and ease of administration, including as compared to alternative treatments and competitive therapies;
- confusion or lack of understanding regarding the effects of rimiducid and the timing and size of dosing of rimiducid after immune cell therapy; and
- the effectiveness of our sales and marketing efforts.

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

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

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

Market acceptance and sales of our product candidates will depend in large part on global reimbursement policies and may be affected by future healthcare reform measures, both in the United States and other key international markets. Patients who are prescribed medicine for the treatment of their conditions generally rely on third-party payors to reimburse all or part of the costs associated with their prescription drugs. Patients are unlikely to use our products unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of our products. Therefore, successful commercialization of our products will depend in part on the availability of governmental and third-party payor reimbursement for the cost of our product candidates and/or payment to the physician for administering our product candidates. In the United States, no uniform policy of coverage and reimbursement for drug products exists among third-party payors. Therefore, coverage and reimbursement for drug products can differ significantly from payor to payor. As a result, the coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our products to each payor separately, with no assurance that coverage and adequate reimbursement will be obtained. One third-party payor's decision to cover a particular medical product or service does not assure that other payors will also provide coverage for the medical product or service, or to provide coverage at an adequate reimbursement rate. As a result, the coverage determination process will require us to provide scientific and clinical support for the use of our products to each payor separately, with no assurance that adequate coverage and reimbursement will be obtained. Further, a third-party payor's decision to provide coverage for a medical product or service does not imply that an adequate reimbursement rate will be approved. The market for our product candidates will depend significantly on access to third-party payors' formularies or lists of treatments for which third-party payors provide coverage and reimbursement. Third party payors may also have difficulty in determining the appropriate coverage of our product candidates, if approved, including rivo-cel, due to the fact that they are combination products that include a small molecule drug, rimiducid. Third-party payors establish coverage and reimbursement policies for new products, including product candidates like rivo-cel and our other product candidates. In particular, in the United States, private health insurers and other third-party payors often provide reimbursement for treatments based on the level at which the government (through the Medicare or Medicaid programs) provides reimbursement for such treatments. In the United States, the EEA and other significant or potentially significant markets for our product candidate, government authorities and third-party payors are increasingly attempting to limit or regulate the price of medical products and services, particularly for new and innovative products and therapies, which has resulted in lower average selling prices. Further, the increased emphasis on managed healthcare in the United States and on country and regional pricing and reimbursement controls in Canada and the EEA will put additional pressure on product pricing, coverage, reimbursement and utilization, which may adversely affect our product sales and results of operations. These pressures can arise from policies and practices of managed care groups, judicial decisions and governmental laws and regulations related to Medicare, Medicaid and healthcare reform, coverage and reimbursement policies and pricing in general. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors.

In March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or collectively, the PPACA, became law in the United States. PPACA substantially changes the way healthcare is financed by both governmental and private insurers and significantly affects the pharmaceutical industry. Among the provisions of the PPACA of greatest importance to the pharmaceutical industry are the following: (i) an annual, nondeductible fee on any entity that manufactures or imports certain branded prescription drugs and biologic agents, apportioned among these entities according to their market share in certain government healthcare programs; (ii) an increase in the rebates a manufacturer must pay under the Medicaid Drug Rebate Program to 23.1% and 13% of the average manufacturer price for branded and generic drugs, respectively; (iii) a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts to negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D; (iv) extension of manufacturers' Medicaid rebate liability to covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations; (v) expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid

coverage to additional individuals with income at or below 133% of the Federal Poverty Level, thereby potentially increasing manufacturers' Medicaid rebate liability; (vi) expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program; (vii) expansion of health care fraud and abuse laws, including the federal civil False Claims Act and the Anti-Kickback Statute, new government investigative powers, and enhanced penalties for noncompliance; and (viii) a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

Since its enactment there have been judicial and Congressional challenges to other aspects of the PPACA, as well as recent efforts by the Trump administration to repeal or replace certain aspects of the PPACA. Since January 2017, President Trump has signed two Executive Orders designed to delay the implementation of certain provisions of the PPACA or otherwise circumvent some of the requirements for health insurance mandated by the PPACA.

Concurrently, Congress has considered legislation that would repeal or repeal and replace all or part of the PPACA. While Congress has not passed comprehensive repeal legislation, two bills affecting the implementation of certain taxes under the PPACA have been signed into law. The Tax Cuts and Jobs Act of 2017 includes a provision repealing, effective January 1, 2019, the tax-based shared responsibility payment imposed by the PPACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the “individual mandate”. Additionally, on January 22, 2018, President Trump signed a continuing resolution on appropriations for fiscal year 2018 that delayed the implementation of certain PPACA-mandated fees, including the so-called “Cadillac” tax on certain high cost employer-sponsored insurance plans, the annual fee imposed on certain health insurance providers based on market share, and the medical device excise tax on non-exempt medical devices. Congress may consider other legislation to replace elements of the PPACA. We continue to evaluate the potential effect of the possible repeal and replacement of the PPACA may have on our business.

In addition, other legislative changes have been proposed and adopted in the United States since the PPACA. For example, through the process created by the Budget Control Act of 2011, there are automatic reductions of Medicare payments to providers up to 2% per fiscal year, which went into effect in April 2013 and, following passage of the Bipartisan Budget Act of 2015, will remain in effect through 2025 unless additional Congressional action is taken. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, further reduced Medicare payments to several providers.

Further, recently there has been heightened governmental scrutiny in the United States over the manner in which drug manufacturers set prices for their marketed products, in light of the rising cost of prescription drugs and biologics. Such scrutiny has resulted in several recent congressional inquiries and proposed federal legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for products. At the state level, legislatures are increasingly passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. We expect that additional federal and state healthcare reform measures will be adopted in the future, any of which could result in reduced demand for our products or other adverse effects on our business.

In the EU, the success of rivo-cel and our other product candidates, if approved, will depend largely on obtaining and maintaining government reimbursement, because in many European countries patients are unlikely to use therapies that are not reimbursed by the government. Negotiating prices with governmental authorities can delay commercialization by 12 months or more. Reimbursement policies may adversely affect our ability to sell our products on a profitable basis. In many international markets, governments control the prices of prescription pharmaceuticals, including through the implementation of reference pricing, price cuts, rebates, revenue-related taxes and profit control, and expect prices of prescription pharmaceuticals to decline over the life of the product or as volumes increase. Recently, many countries in the EEA have increased the amount of discounts required on pharmaceutical products and other therapies, and we expect these discounts to continue as countries attempt to manage healthcare expenditures, especially in light of current economic conditions. As a result of these pricing practices, it may become difficult to achieve profitability or expected rates of growth in revenue or results of operations. Any shortfalls in revenue could adversely affect our business, prospects, financial condition and results of operations.

Certain countries have a very difficult reimbursement environment and we may not obtain reimbursement or pricing approval, if required, in all countries where we expect to market a product, or we may obtain reimbursement approval at a level that would make marketing a product in certain countries not viable.

We expect to experience pricing pressures in connection with the sale of rivo-cel and rimiducid, if approved, and any other products that we may develop, due to the trend toward managed healthcare, the increasing influence of health

maintenance organizations and additional legislative proposals. If we fail to successfully secure and maintain adequate coverage and reimbursement for our products or are significantly delayed in doing so, we will have difficulty achieving market acceptance of our products and expected revenue and profitability which would have a material adverse effect on our business, prospects, financial condition and results of operations.

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

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

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

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

We are subject to U.S. and certain foreign export and import controls, sanctions, embargoes, anti-corruption laws, and anti-money laundering laws and regulations. Compliance with these legal standards could impair our ability to compete in domestic and international markets. We can face criminal liability and other serious consequences for violations, which can harm our business.

We are subject to export control and import laws and regulations, including the U.S. Export Administration Regulations, U.S. Customs regulations, various economic and trade sanctions regulations administered by the U.S. Treasury Department's Office of Foreign Assets Controls, the U.S. Foreign Corrupt Practices Act of 1977, as amended, or FCPA, the U.S. domestic bribery statute contained in 18 U.S.C. § 201, the U.S. Travel Act, the USA PATRIOT Act, and other state and national anti-bribery and anti-money laundering laws in the countries in which we conduct activities. Anti-corruption laws are interpreted broadly and prohibit companies and their employees, agents, contractors, and other collaborators from authorizing, promising, offering, or providing, directly or indirectly, improper payments or anything else of value to recipients in the public or private sector. We may engage third parties for clinical trials outside of the United States, to sell our products abroad once we enter a commercialization phase, and/or to obtain necessary permits, licenses, patent registrations, and other regulatory approvals. We have direct or indirect interactions with officials and employees of government agencies or government-affiliated hospitals, universities, and other organizations. We can be held liable for the corrupt or other illegal activities of our employees, agents, contractors, and other collaborators, even if we do not explicitly authorize or have actual knowledge of such activities. Any violations of the laws and regulations described above may result in substantial civil and criminal fines and penalties, imprisonment, the loss of export or import privileges, debarment, tax reassessments, breach of contract and fraud litigation, reputational harm, and other consequences.

### Risks Related to Our Intellectual Property

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

We are dependent on patents, know-how and proprietary technology, both our own and licensed from others. We license from Baylor College of Medicine, or Baylor, certain intellectual property related to methods for activating antigen presenting cells, to certain genetic constructs and to certain methods for inducing apoptosis. Baylor may terminate or modify our licenses in the event of a material breach by us that remains uncured following the date that is 90 days after written notice of such breach or upon certain insolvency events that remain uncured following the date that is 30 days following written notice of such insolvency event. In addition, we have funded certain of our ongoing clinical development and will fund certain of our future clinical development with funds from the State of Texas. The State of Texas may have rights to commercialize the results of those clinical trials if it determines that we have failed, after notice and an opportunity to cure, to use diligent and commercially reasonable efforts to commercialize or otherwise bring to practical application the results of the funded clinical trials. We are also dependent on our license agreements with Agensys, Inc. (a subsidiary of Astellas Pharma, Inc.) with respect to PSCA-targeted CARs, Leiden University with respect to certain TCRs and BioVec Pharma Inc. with respect to making retrovirus for all of our programs. The termination of any of these licenses could have a material adverse effect on our business.

Any termination of these agreements, or other agreements to which we are a party could result in the loss of significant rights and could harm our ability to commercialize our product candidates. See “Item 1. Business—Our License Agreements” for additional information regarding our license agreements.

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

- the scope of rights granted under the license agreement and other interpretation-related issues;
- whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- our right to sublicense patent and other rights to third parties under collaborative development relationships;
- our diligence obligations with respect to the use of the licensed technology in relation to our development and commercialization of our product candidates, and what activities satisfy those diligence obligations; and
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners.

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

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

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

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

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

- if and when patents will issue;
- the degree and range of protection any issued patents will afford us against competitors including whether third parties will find ways to invalidate or otherwise circumvent our patents;
- whether or not others will obtain patents claiming aspects similar to those covered by our patents and patent applications; or





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

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

Patent coverage on the dimerization molecule rimiducid, expired in February 2016. Therefore, any additional barriers to entry for competitors to use rimiducid may not be effective in preventing such use. There remain significant questions regarding how the FDA will interpret the ‘biosimilar’ provisions recently added to the PHSA as applied to complex biological products such as our investigational products. Depending on how the FDA ultimately interprets these provisions, if our investigational products incorporating rimiducid receive FDA approval through a combination product BLA, then a biosimilar of these combination products could be approved by the FDA twelve years from the date that we receive FDA approval for our application. In addition, if a third party were able to obtain FDA approval of a new drug application for rimiducid on its own, then it is possible that other third parties could later seek approval of an abbreviated new drug application for rimiducid.

We rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable, processes for which patents are difficult to enforce and any other elements of our product discovery and development processes that involve proprietary know-how, information, or technology that is not covered by patents. We require all of our employees to assign their inventions to us, and require all of our employees, consultants, advisors and any third parties who have access to our proprietary know-how, information, or technology to enter into confidentiality agreements; however, it is possible that our trade secrets and other confidential proprietary information could be disclosed or that competitors may otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. Furthermore, the laws of some foreign countries do not protect proprietary rights to the same extent or in the same manner as the laws of the U.S. As a result, we may encounter significant problems in protecting and defending our intellectual property both in the U.S. and abroad. If we are

unable to prevent unauthorized material disclosure of our intellectual property to third parties, we will not be able to establish or maintain a competitive advantage in our market, which could materially adversely affect our business, operating results and financial condition.

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

Our commercial success depends in part on our avoiding infringement of the patents and proprietary rights of third parties. There is a substantial amount of litigation involving patents and other intellectual property rights in the biotechnology and pharmaceutical industries, as well as administrative proceedings for challenging patents, including interference and reexamination proceedings

before the USPTO or oppositions and other comparable proceedings in foreign jurisdictions. Recently, under U.S. patent reform, new procedures including inter parties review and post grant review have been implemented. As stated above, this reform is untried and untested and will bring uncertainty to the possibility of challenge to our patents in the future. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are developing our product candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may give rise to claims of infringement of the patent rights of others.

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

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

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

We are aware of third-party patents having claims that may be considered as being directed to single-chain antibody fragments that bind to PSCA and these patents may be considered relevant to BPX-601 and related technologies we are developing. We currently are evaluating whether or not we need to obtain rights to these patents under a license, and if it is determined that we need to obtain such rights, whether these rights can be obtained. We are also aware of third-party patent applications having claims that may be considered as being directed to cellular therapy constructs utilizing a heterodimer domain for activation of iC9. We are monitoring these applications and if they are granted with the claims as drafted they may be relevant to our potential dual-switch product candidates containing such a heterodimer activation domain.

Also, while we are aware there are other third-party patents having claims that may be considered relevant to technologies for which we are seeking, or plan to seek, regulatory approval, we believe those patents have a patent

term that may expire prior to the time we expect to obtain regulatory approval for these technologies. The estimated expiration dates for those patents were determined according to information on the face pages of the patents, and certain factors that could influence patent term, such as patent term adjustment and patent term extension, for example, were not factored into these estimates. Accordingly, the estimated expiration dates of those patents may not be accurate and one or more of those patents may not expire before we obtain regulatory approval for an applicable technology. Owners or licensees of one or more of those patents may bring a patent infringement suit against us. If one or more of those patents are asserted against us, we may be able to assert a defense for a safe harbor to patent infringement under 35 U.S.C. 271(e)(1) if certain requirements are met. It is possible that (1) certain of these requirements may not be met, and/or (2) one or more of the third-party patents might expire after one or more of our technologies obtain regulatory

approval, and consequently we may not successfully assert such a defense to patent infringement. If we are unsuccessful in asserting a defense under 35 U.S.C. 271(e)(1), it is possible we may not prevail in defending against claims of infringement and/or challenging the validity of claims in those patents. We may not successfully develop alternative technologies or enter into agreements by which we obtain rights to applicable patents. These rights, if necessary, may not be available on terms acceptable to us.

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

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

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

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

We may not be able to successfully complete negotiations and ultimately acquire the rights to the intellectual property that we may seek to acquire in the future.

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

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

Interference proceedings provoked by third parties or brought by the USPTO may be necessary to determine the priority of inventions with respect to our patents or patent applications or those of our licensors. An unfavorable outcome could result in a loss of our patent rights and could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. It also is possible that third parties could institute a patent office post-grant proceeding against one or more of our patents, or one or more patents licensed to us, such as a post grant review proceeding, inter parties review proceeding or reexamination proceeding at the USPTO, or an opposition proceeding in a jurisdiction outside the U.S. An unfavorable outcome in a post-grant proceeding could result in a loss of our patent rights. Litigation, interference proceedings or patent office post-grant proceedings may result in a decision adverse to our interests and, even if we are successful, may result in substantial costs and distract our management and other employees. We also may not be able to prevent, alone or with our licensors, misappropriation of our trade secrets or confidential information, particularly in countries where the laws may not protect those rights as fully as in the U.S.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities

analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock.

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

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

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

If we or one of our licensing partners initiate legal proceedings against a third party to enforce a patent covering one of our product candidates, the defendant could counterclaim that the patent covering our product candidate, as applicable, is invalid and/or unenforceable. In patent litigation in the U.S., defendant counterclaims alleging invalidity and/or unenforceability are commonplace, and there are numerous grounds upon which a third party can assert invalidity or unenforceability of a patent. Third parties may also raise similar claims before administrative bodies in the U.S. or abroad, even outside the context of litigation. Such mechanisms include re-examination, post grant review, and equivalent proceedings in foreign jurisdictions, for example, opposition proceedings. Any such proceedings could result in revocation or amendment to our patents in such a way that they no longer cover our product candidates. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art and that prior art that was cited during prosecution, but not relied on by the patent examiner, will not be revisited. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we would lose at least part, and perhaps all, of the patents directed to our product candidates. A loss of patent rights could have a material adverse impact on our business.

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

As is the case with other biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involve both technological and legal complexity, and is therefore costly, time-consuming and inherently uncertain. In addition, the U.S. has recently enacted and is currently implementing wide-ranging patent reform legislation. Recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained.

Depending on decisions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future. For example, in the recent case, *Assoc. for Molecular Pathology v. Myriad Genetics, Inc.*, the U.S. Supreme Court held that certain claims to DNA molecules are not patentable. While we do not believe that any of the patents owned or licensed by us will be found invalid based on this decision, we cannot predict how future decisions by the courts, the U.S. Congress or the USPTO may impact the value of our patents.





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

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

Many companies have encountered significant problems in protecting and defending intellectual property in foreign jurisdictions. The legal systems of certain countries, particularly China and certain other developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property, particularly those relating to biopharmaceutical products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. To date, we have not sought to enforce any issued patents in these foreign jurisdictions. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate, and the damages or other remedies awarded, if any, may not be commercially meaningful. The requirements for patentability may differ in certain countries, particularly developing countries. Furthermore, generic drug manufacturers or other competitors may challenge the scope, validity or enforceability of our or our licensors' patents, requiring us or our licensors to engage in complex, lengthy and costly litigation or other proceedings. Certain countries in Europe and developing countries, including China and India, have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In those countries, we and our licensors may have limited remedies if patents are infringed or if we or our licensors are compelled to grant a license to a third party, which could materially diminish the value of those patents. This could limit our potential revenue opportunities. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

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

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

### Risks Related to Ownership of our Common Stock

We are subject to securities litigation, which is expensive and could divert management attention.\*

Our share price has been and may continue to be volatile. Companies that have experienced volatility in the market price of their stock have been subject to securities class action litigation. We are a target of this type of litigation. For example, on February 6, 2018, a purported securities class action complaint captioned *Nipun Kakkar v. Bellicum Pharmaceuticals, Inc., Rick Fair and Alan Musso* was filed against us, and certain of our officers in the U.S. District Court for the Southern District of Texas, Houston Division. A second substantially similar class action was filed on March 14, 2018 by plaintiff Frances Rudy against the same defendants in the same court. The lawsuits purport to assert class action claims on behalf of purchasers of our securities during the period from May 8, 2017 through January 30, 2018. The complaints allege that the defendants violated the Exchange Act by making materially false and misleading statements concerning our clinical trials being conducted in the U.S. to assess rivo-cel as an adjunct T-cell therapy administered after allogeneic hematopoietic stem cell transplantation. The complaints purport to assert claims for violations of Sections 10(b) and 20(a) of the Exchange Act and Rule 10b-5 promulgated thereunder. The complaints seek, on behalf of the purported class, an unspecified amount of monetary damages, interest, fees and expenses of attorneys and experts, and other relief. On April 9, 2018, the District Court consolidated the two lawsuits under the Kakkar action and motions were filed by putative class members for appointment as lead plaintiff and approval of lead counsel. The District Court has yet to rule on the motions.

On July 19, 2018, a purported shareholder derivative complaint captioned *Seung Paik v. Richard A. Fair, et al.* was filed against the Company's directors and certain of the Company's officers in the U.S. District Court for the Southern District of Texas, Houston Division. The lawsuit purports to seek damages on behalf of the Company against the individual defendants for breach of fiduciary duty, waste, unjust enrichment and violations of Section 14(a) of the Exchange Act. The complaint alleges that the defendants caused or allowed the Company to disseminate misstatements regarding the clinical trials for rivo-cel and to make false or misleading statements in the proxy materials for the Company's 2017 annual meeting of stockholders. On October 3, 2018, the District Court granted the Company's motion to stay the derivative cause of action until reinstated on motion of the parties.

Litigation of this type could result in substantial costs and diversion of management's attention and resources, which could adversely impact our business. Any adverse determination in litigation could also subject us to significant liabilities.

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

Prior to our December 2014 IPO, there was no public market for our common stock. The trading price of our common stock is likely to continue to be highly volatile and subject to wide fluctuations in response to various factors, some of which are beyond our control, including market conditions in general and a limited trading volume for our shares. In addition to the factors discussed in this "Risk Factors" section and elsewhere in our Annual Report, these factors include:

- the commencement, enrollment or results of the planned clinical trials of our product candidates or any future clinical trials we may conduct, or changes in the development status of our product candidates;
- any delay in our regulatory filings for our product candidates and any adverse development or perceived adverse development with respect to the applicable regulatory authority's review of such filings, including without limitation the FDA's issuance of a "refusal to file" letter or a request for additional information;
- adverse results or delays in our ongoing or future clinical trials, including for rivo-cel;
- our decision to initiate a clinical trial, not to initiate a clinical trial or to terminate an existing clinical trial;
- adverse regulatory decisions, including failure to receive regulatory approval of our product candidates;
- changes in laws or regulations applicable to our products, including but not limited to clinical trial requirements for approvals;
- adverse developments concerning our CID technology platform and our small molecule drug rimiducid;
- adverse developments concerning our manufacturers;
- our inability to obtain adequate product supply for any approved product or inability to do so at acceptable prices;
- our inability to maintain successful collaborations or to establish new collaborations if needed;
- our failure to commercialize our product candidates;
- additions or departures of key scientific or management personnel;

- unanticipated serious safety concerns related to the use of our product candidates;
- introduction of new products or services offered by us or our competitors;

57

---

announcements of significant acquisitions, strategic partnerships, joint ventures or capital commitments by us or our competitors;

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

In addition, the stock market in general, and The Nasdaq Global Market and biopharmaceutical companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance. In the past, securities class action litigation has often been instituted against companies following periods of volatility in the market price of a company's securities. This type of litigation, if instituted, could result in substantial costs and a diversion of management's attention and resources, which would harm our business, operating results or financial condition.

We do not intend to pay dividends on our common stock, so any returns will be limited to the value of our stock. We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. In addition, the terms of our loan and security agreement with Oxford restrict our ability to declare or pay any cash dividend or make a cash distribution on any class of stock or other equity interest. Any return to stockholders will therefore be limited to the appreciation of their stock.

Our principal stockholders and management own a significant percentage of our stock and can exert significant control over matters subject to stockholder approval.\*

As of October 31, 2018, our executive officers, directors and 5% stockholders beneficially owned approximately 27.9% of our outstanding voting shares. Therefore, these stockholders may have the ability to significantly influence us through this ownership position. These stockholders may be able to significantly influence all matters requiring stockholder approval. For example, these stockholders may be able to significantly influence elections of directors, amendments of our organizational documents, or approval of any merger, sale of assets, or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may feel are in your best interest as one of our stockholders.

We are an emerging growth company and the reduced reporting requirements applicable to emerging growth companies may make our common stock less attractive to investors.

We are an emerging growth company, as defined in the JOBS Act enacted in April 2012. For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, as amended, or the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in our Annual Report and our periodic reports and proxy statements and exemptions from the requirements of holding nonbinding advisory votes on executive compensation and stockholder approval of any golden parachute payments not previously approved. We could be an emerging growth company through 2019, although circumstances could cause us to lose that status earlier. We will remain an emerging growth company until the earlier of (a) December 31, 2019, (b) the last day of the fiscal year in which we have total annual gross revenue of at least \$1.0 billion, (c) the last day of the fiscal year in which we are deemed to be a large accelerated filer, which requires the market value of our common stock that is held by non-affiliates to exceed \$700.0 million as of the prior September 30th, or (d) the date on which we have issued more than \$1.0 billion in non-convertible debt during the prior three-year period.

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

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

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

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

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

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

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

We expect that significant additional capital will be needed in the future to continue our planned operations, including conducting clinical trials, commercialization efforts for rivo-cel, expanded research and development activities and costs associated with operating a public company. To raise capital, we may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time, including pursuant to our shelf registration statement on Form S-3 that we filed with the SEC. In addition, on October 5, 2018, we entered into an Open Market Sale Agreement with Jefferies LLC, as sales agent, pursuant to which we may offer and sell, from time to time, shares of common stock with an aggregate offering price of up to \$60.0 million. If we sell common stock, convertible securities or other equity securities, investors may be materially diluted by

subsequent sales. Any such sales may also result in material dilution to our existing stockholders, and new investors could gain rights, preferences and privileges senior to the existing holders of our common stock.

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

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

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

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

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

The trading market for our common stock depends in part on the research and reports that securities or industry analysts publish about us or our business. In the event securities or industry analysts that cover us downgrade our stock or publish inaccurate or unfavorable research about our business, our stock price may decline. If one or more of these analysts ceases coverage of us or fails to publish reports on us regularly, demand for our stock could decrease, which might cause our stock price and trading volume to decline.

## Item 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

None.

### Purchase of Equity Securities

Table of Contents

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

Item 3. Defaults Upon Senior Securities

None.

Item 4. Mine Safety Disclosures

Not applicable.

Item 5. Other Information

None.

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.

EXHIBIT

INDEX

Exhibit number	Description of exhibit
3.1 <sup>(1)</sup>	<u>Amended and Restated Certificate of Incorporation of the Registrant.</u>
3.2 <sup>(2)</sup>	<u>Amended and Restated Bylaws of the Registrant.</u>
4.1	Reference is made to Exhibits 3.1 and 3.2.
4.2 <sup>(3)</sup>	<u>Form of Common Stock Certificate of the Registrant.</u>
4.3 <sup>(4)</sup>	<u>Second Amended and Restated Investor Rights Agreement by and among the Registrant and certain of its stockholders, dated August 22, 2014.</u>
4.4 <sup>(5)</sup>	<u>Registration Rights Agreement by and among the Registrant and Baker Brothers Life Sciences, LP, and two of its affiliated funds, dated January 15, 2016.</u>
10.1 <sup>(6)+</sup>	<u>Separation Agreement by and between Registrant and Alan Musso, dated July 13, 2018.</u>
10.2 <sup>(7)+</sup>	<u>Retention Agreement by and between Registrant and Rosemary Williams, dated July 17, 2018.</u>
10.3 <sup>(8)+</sup>	<u>Open Market Sale Agreement by and between Registrant and Jefferies LLC, dated October 5, 2018.</u>
31.1	<u>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.</u>
31.2	<u>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.</u>
32.1	<u>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.</u>
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document





Table of Contents

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
+	Indicates management contract or compensatory plan.
(1)	Incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K, filed with the SEC on December 23, 2014.
(2)	Incorporated by reference to Exhibit 3.2 to the Registrant's Current Report on Form 8-K, filed with the SEC on December 23, 2014.
(3)	Incorporated by reference to Exhibit 4.1 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.
(4)	Incorporated by reference to Exhibit 4.2 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.
(5)	Incorporated by reference to Exhibit 4.4 to the Registrant's Annual Report on Form 10-K, filed with the SEC on March 14, 2016.
(6)	Incorporated by reference to Exhibit 10.1 to the Registrant's Quarterly Report on Form 10-Q, filed with the SEC on August 7, 2018.
(7)	Incorporated by reference to Exhibit 10.2 to the Registrant's Quarterly Report on Form 10-Q, filed with the SEC on August 7, 2018.
(8)	Incorporated by reference to the Registrant's Current Report on Form 8-K, filed with the SEC on October 5, 2018.

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: November 6, 2018 By: /s/ Richard A. Fair  
Richard A. Fair  
President and Chief Executive Officer

Date: November 6, 2018 By: /s/ Rosemary Y. Williams  
Rosemary Y. Williams  
Principal Accounting Officer