

**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
Washington, D.C. 20549**

**FORM 10-Q**

**(Mark One)**

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

**For the quarterly period ended June 30, 2019**

**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: 001-35403**

**Verastem, Inc.**

(Exact name of registrant as specified in its charter)

**Delaware**

(State or other jurisdiction of  
incorporation or organization)

**117 Kendrick Street, Suite 500**

**Needham, MA**

(Address of principal executive offices)

**27-3269467**

(I.R.S. Employer  
Identification Number)

**02494**

(Zip Code)

**(781) 292-4200**

(Registrant's telephone number, including area code)

Securities registered pursuant to Section 12(b) of the Securities Exchange Act of 1934:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, \$0.0001 par value per share	VSTM	The Nasdaq Global Market

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 (§232.405 of this chapter) 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, 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.

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

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

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

As of August 1, 2019, there were 74,140,687 shares of Common Stock outstanding.

**TABLE OF CONTENTS**

<b><u>PART I—FINANCIAL INFORMATION</u></b>	
<a href="#">Item 1. Condensed Consolidated Financial Statements (unaudited)</a>	4
<a href="#">Item 2. Management’s Discussion and Analysis of Financial Condition and Results of Operations</a>	28
<a href="#">Item 3. Quantitative and Qualitative Disclosures About Market Risk</a>	42
<a href="#">Item 4. Controls and Procedures</a>	43
<b><u>PART II—OTHER INFORMATION</u></b>	
<a href="#">Item 1. Legal Proceedings</a>	44
<a href="#">Item 1A. Risk Factors</a>	44
<a href="#">Item 2. Unregistered Sales of Equity Securities and Use of Proceeds</a>	44
<a href="#">Item 3. Defaults Upon Senior Securities</a>	45
<a href="#">Item 4. Mine Safety Disclosures</a>	45
<a href="#">Item 5. Other Information</a>	45
<a href="#">Item 6. Exhibits</a>	45
<a href="#">EXHIBIT INDEX</a>	46
<a href="#">SIGNATURES</a>	47

## FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements related to present facts or current conditions or historical facts, contained in this Quarterly Report on Form 10-Q, including statements regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management, are forward-looking statements. Such statements relate to, among other things, the development and activity of our lead product, COPIKTRA™ and our Phosphoinositide 3-kinase (PI3K) and Focal Adhesion Kinase (FAK) programs generally, the potential commercial success of COPIKTRA, the anticipated adoption of COPIKTRA by patients and physicians, the structure of our planned and pending clinical trials, and the timeline and indications for clinical development, regulatory submissions and commercialization of activities. The words “anticipate,” “believe,” “estimate,” “expect,” “intend,” “may,” “plan,” “predict,” “project,” “target,” “potential,” “will,” “would,” “could,” “should,” “continue” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

Forward-looking statements are not guarantees of future performance and our actual results could differ materially from the results discussed in the forward-looking statements we make. Applicable risks and uncertainties include the risks and uncertainties, among other things, regarding: the commercial success of COPIKTRA in the United States; physician and patient adoption of COPIKTRA, including those related to the safety and efficacy of COPIKTRA; the uncertainties inherent in research and development of COPIKTRA, such as negative or unexpected results of clinical trials; whether and when any applications for COPIKTRA may be filed with regulatory authorities in any other jurisdictions; whether and when regulatory authorities in any other jurisdictions may approve any such other applications that may be filed for COPIKTRA, which will depend on the assessment by such regulatory authorities of the benefit-risk profile suggested by the totality of the efficacy and safety information submitted and, if approved, whether COPIKTRA will be commercially successful in such jurisdictions; our ability to obtain, maintain and enforce patent and other intellectual property protection for COPIKTRA and our other product candidates; the scope, timing, and outcome of any legal proceedings; decisions by regulatory authorities regarding labeling and other matters that could affect the availability or commercial potential of COPIKTRA; the fact that regulatory authorities in the U.S. or other jurisdictions, if approved, could withdraw approval; whether preclinical testing of our product candidates and preliminary or interim data from clinical trials will be predictive of the results or success of ongoing or later clinical trials; that the timing, scope and rate of reimbursement for our product candidates is uncertain; that third-party payors (including government agencies) may not reimburse for COPIKTRA; that there may be competitive developments affecting our product candidates; that data may not be available when expected; that enrollment of clinical trials may take longer than expected; that COPIKTRA or our other product candidates will cause unexpected safety events, experience manufacturing or supply interruptions or failures, or result in unmanageable safety profiles as compared to their levels of efficacy; that COPIKTRA will be ineffective at treating patients with lymphoid malignancies; that we will be unable to successfully initiate or complete the clinical development and eventual commercialization of our product candidates; that the development and commercialization of our product candidates will take longer or cost more than planned; that we may not have sufficient cash to fund our contemplated operations; that we, CSPC Pharmaceutical Group, Yakult Honsha Co., Ltd., Sanofi or Infinity Pharmaceuticals, Inc. will fail to fully perform under the duvelisib license agreements; that we may be unable to make additional draws under our debt facility or obtain adequate financing in the future through product licensing, co-promotional arrangements, public or private equity, debt financing or otherwise; that we will not pursue or submit regulatory filings for our product candidates, including for duvelisib in patients with chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL) or indolent non-Hodgkin lymphoma (iNHL) in other jurisdictions; and that our product candidates will not receive regulatory approval, become commercially successful products, or result in new treatment options being offered to patients. Other risks and uncertainties include those identified in our Annual Report on Form 10-K for the year ended December 31, 2018 as filed with the Securities and Exchange Commission (SEC) on March 12, 2019, and in any subsequent filings with the SEC.

As a result of these and other factors, we may not achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments we may make. The forward-looking statements contained in this Quarterly Report on Form 10-Q reflect our views as of the date hereof. We do not assume and specifically disclaim any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by law.

**PART I—FINANCIAL INFORMATION****Item 1. Condensed Consolidated Financial Statements (unaudited).**

**Verastem, Inc.**  
**CONDENSED CONSOLIDATED BALANCE SHEETS**  
(in thousands, except per share amounts)

	June 30, 2019	December 31, 2018
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 113,080	\$ 129,867
Short-term investments	74,173	119,786
Accounts receivable, net	1,389	306
Inventory	294	327
Prepaid expenses and other current assets	3,410	2,973
Total current assets	192,346	253,259
Property and equipment, net	1,149	1,369
Right-of-use asset, net	3,225	—
Intangible assets, net	20,793	21,577
Other assets	1,028	1,031
Total assets	<u>\$ 218,541</u>	<u>\$ 277,236</u>
<b>Liabilities and stockholders' equity</b>		
Current liabilities:		
Accounts payable	\$ 11,538	\$ 10,253
Accrued expenses	19,372	21,108
Lease liability, short-term	294	—
Current portion of long-term debt	—	5,716
Total current liabilities	31,204	37,077
Non-current liabilities:		
Long-term debt	34,673	19,506
Convertible senior notes	99,163	95,231
Lease liability, long-term	3,694	—
Other non-current liabilities	500	1,123
Total liabilities	169,234	152,937
Stockholders' equity:		
Preferred stock, \$0.0001 par value; 5,000 shares authorized, no shares issued and outstanding at June 30, 2019 and December 31, 2018, respectively	—	—
Common stock, \$0.0001 par value; 200,000 shares authorized, 73,877 and 73,806 shares issued and outstanding at June 30, 2019 and December 31, 2018, respectively	7	7
Additional paid-in capital	505,086	499,741
Accumulated other comprehensive income	86	127
Accumulated deficit	(455,872)	(375,576)
Total stockholders' equity	49,307	124,299
Total liabilities and stockholders' equity	<u>\$ 218,541</u>	<u>\$ 277,236</u>

See accompanying notes to the condensed consolidated financial statements.

**Verastem, Inc.**  
**CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS**  
**(unaudited)**  
**(in thousands, except per share amounts)**

	<u>Three months ended June 30,</u>		<u>Six months ended June 30,</u>	
	<u>2019</u>	<u>2018</u>	<u>2019</u>	<u>2018</u>
Revenue:				
Product revenue, net	\$ 3,019	\$ —	\$ 4,690	\$ —
License and collaboration revenue	117	10,000	117	10,000
<b>Total revenue</b>	<b>3,136</b>	<b>10,000</b>	<b>4,807</b>	<b>10,000</b>
Operating expenses:				
Cost of sales - product	377	—	534	—
Cost of sales - intangible amortization	392	—	785	—
Research and development	11,346	12,381	21,103	23,315
Selling, general and administrative	29,298	15,813	55,331	25,640
<b>Total operating expenses</b>	<b>41,413</b>	<b>28,194</b>	<b>77,753</b>	<b>48,955</b>
Loss from operations	(38,277)	(18,194)	(72,946)	(38,955)
Interest income	1,268	343	2,765	534
Interest expense	(5,185)	(516)	(10,115)	(996)
<b>Net loss</b>	<b>\$ (42,194)</b>	<b>\$ (18,367)</b>	<b>\$ (80,296)</b>	<b>\$ (39,417)</b>
Net loss per share—basic and diluted	\$ (0.57)	\$ (0.30)	\$ (1.09)	\$ (0.70)
Weighted average common shares outstanding used in computing net loss per share—basic and diluted	73,877	61,256	73,865	56,074
<b>Net loss</b>	<b>\$ (42,194)</b>	<b>\$ (18,367)</b>	<b>\$ (80,296)</b>	<b>\$ (39,417)</b>
Unrealized (loss) gain on available-for-sale securities	(24)	4	(41)	6
<b>Comprehensive loss</b>	<b>\$ (42,218)</b>	<b>\$ (18,363)</b>	<b>\$ (80,337)</b>	<b>\$ (39,411)</b>

See accompanying notes to the condensed consolidated financial statements.

**Verastem, Inc.**  
**CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY**  
**(unaudited)**  
**(in thousands, except share data)**

	Common stock		Additional paid-in capital	Accumulated other comprehensive (loss) income	Accumulated deficit	Total stockholders' equity
	Shares	Amount				
<b>Balance at December 31, 2018</b>	<b>73,806,344</b>	<b>\$ 7</b>	<b>\$ 499,741</b>	<b>\$ 127</b>	<b>\$ (375,576)</b>	<b>\$ 124,299</b>
Net loss	—	—	—	—	(38,102)	(38,102)
Unrealized loss on available-for-sale marketable securities	—	—	—	(17)	—	(17)
Issuance of common stock resulting from exercise of stock options	46,803	—	75	—	—	75
Issuance of common stock resulting from vesting of restricted stock units and payment of tax withholdings	23,792	—	(43)	—	—	(43)
Stock-based compensation expense	—	—	2,248	—	—	2,248
<b>Balance at March 31, 2019</b>	<b>73,876,939</b>	<b>\$ 7</b>	<b>\$ 502,021</b>	<b>\$ 110</b>	<b>\$ (413,678)</b>	<b>\$ 88,460</b>
Net loss	—	—	—	—	(42,194)	(42,194)
Unrealized (loss) on available-for-sale marketable securities	—	—	—	(24)	—	(24)
Stock-based compensation expense	—	—	3,065	—	—	3,065
<b>Balance at June 30, 2019</b>	<b>73,876,939</b>	<b>\$ 7</b>	<b>\$ 505,086</b>	<b>\$ 86</b>	<b>\$ (455,872)</b>	<b>\$ 49,307</b>
<b>Balance at December 31, 2017</b>	<b>50,800,908</b>	<b>\$ 5</b>	<b>\$ 360,823</b>	<b>\$ (2)</b>	<b>\$ (303,142)</b>	<b>\$ 57,684</b>
Net loss	—	—	—	—	(21,050)	(21,050)
Unrealized gain on available-for-sale marketable securities	—	—	—	2	—	2
Issuance of common stock resulting from at-the-market transactions, net of issuance costs of \$0	167,065	—	588	—	—	588
Stock-based compensation expense	—	—	1,328	—	—	1,328
<b>Balance at March 31, 2018</b>	<b>50,967,973</b>	<b>\$ 5</b>	<b>\$ 362,739</b>	<b>\$ —</b>	<b>\$ (324,192)</b>	<b>\$ 38,552</b>
Net loss	—	—	—	—	(18,367)	(18,367)
Unrealized (loss) on available-for-sale marketable securities	—	—	—	4	—	4
Issuance of common stock resulting from at-the-market transactions, net of issuance costs of \$0	6,314,410	1	23,687	—	—	23,688
Issuance of common stock resulting from follow-on offering, net of issuance costs of \$361	16,111,110	1	81,188	—	—	81,189
Issuance of common stock resulting from exercise of stock options	186,206	—	261	—	—	261
Stock-based compensation expense	—	—	1,540	—	—	1,540
<b>Balance at June 30, 2018</b>	<b>73,579,699</b>	<b>\$ 7</b>	<b>\$ 469,415</b>	<b>\$ 4</b>	<b>\$ (342,559)</b>	<b>\$ 126,867</b>

See accompanying notes to the condensed consolidated financial statements.

**Verastem, Inc.**  
**CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS**  
**(unaudited)**  
**(in thousands)**

	<b>Six months ended June 30,</b>	
	<b>2019</b>	<b>2018</b>
<b>Operating activities</b>		
Net loss	\$ (80,296)	\$ (39,417)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation	227	792
Amortization of acquired intangible asset	785	—
Amortization of right-of-use asset and lease liability	112	—
Stock-based compensation expense	5,313	2,867
Amortization of deferred financing costs, debt discounts and premiums and discounts on available-for-sale marketable securities	2,815	178
Gain on sale of fixed assets	—	(79)
Changes in operating assets and liabilities:		
Accounts receivable, net	(1,083)	—
Inventory	33	—
Prepaid expenses, other current assets and other assets	(434)	(457)
Accounts payable	1,278	(1,436)
Accrued expenses and other liabilities	(2,199)	4,785
Net cash used in operating activities	(73,449)	(32,767)
<b>Investing activities</b>		
Purchases of property and equipment	—	(677)
Sales of property and equipment	—	82
Purchases of investments	(37,637)	—
Maturities of investments	84,530	4,500
Net cash provided by investing activities	46,893	3,905
<b>Financing activities</b>		
Proceeds from long-term debt, net of issuance costs	9,694	9,900
Proceeds from the exercise of stock options	75	262
Proceeds from the issuance of common stock, net	—	105,457
Net cash provided by financing activities	9,769	115,619
Increase (decrease) in cash, cash equivalents and restricted cash	(16,787)	86,757
Cash, cash equivalents and restricted cash at beginning of period	130,608	82,338
Cash, cash equivalents and restricted cash at end of period	\$ 113,821	\$ 169,095
<b>Supplemental disclosure of non-cash investing and financing activities</b>		
Purchases of property and equipment in accounts payable	\$ 7	\$ 527
Common stock issuance costs included in accounts payable and accrued expenses	\$ 15	\$ 316

See accompanying notes to the condensed consolidated financial statements.

**Verastem, Inc.**  
**NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS**  
**(unaudited)**

**1. Nature of business**

Verastem, Inc. (the Company) is a biopharmaceutical company focused on developing and commercializing medicines to improve the survival and quality of life of cancer patients. On September 24, 2018, the Company's first commercial product, COPIKTRA™ (duvelisib), was approved by the U.S. Food and Drug Administration (the FDA) for the treatment of patients with certain hematologic cancers including chronic lymphocytic leukemia (CLL), small lymphocytic lymphoma (SLL) and follicular lymphoma (FL). Both its marketed product, COPIKTRA, and most advanced product candidate, defactinib, utilize a multi-faceted approach designed to treat cancers originating either in the blood or major organ systems. The Company is currently developing its product candidates in both preclinical and clinical studies as potential therapies for certain cancers, including leukemia, lymphoma, lung cancer, ovarian cancer, colorectal cancer, head and neck cancer, mesothelioma, and pancreatic cancer. The Company believes that these compounds may be beneficial as therapeutics either as single agents or when used in combination with immuno-oncology agents or other current and emerging standard of care treatments in aggressive cancers that do not adequately respond to currently available therapies.

The consolidated financial statements include the accounts of Verastem Securities Company, a wholly-owned subsidiary of the Company. All financial information presented has been consolidated and includes the accounts of the Company and its wholly-owned subsidiary. All intercompany balances and transactions have been eliminated in consolidation. On July 11, 2019 the Company completed the registration of Verastem Europe GmbH, wholly owned subsidiary.

As of June 30, 2019, the Company had cash, cash equivalents and short-term investments of \$187.3 million and accumulated deficit of \$455.9 million. The Company is subject to the risks associated with other life science companies, including, but not limited to, possible failure of preclinical testing or clinical trials, competitors developing new technological innovations, market acceptance and the commercial success of COPIKTRA, or any of the Company's investigational product candidates following receipt of regulatory approval, protection of proprietary technology and the continued ability to obtain adequate financing to fund the Company's future operations. If the Company does not successfully commercialize COPIKTRA or any of its other product candidates, it will be unable to generate product revenue or achieve profitability and may need to raise additional capital.

The Company has historical losses from operations and anticipates that it will continue to incur losses as it continues the commercialization of COPIKTRA and the research and development of its product candidates. The Company believes that it may have sufficient funds to meet its obligations within the next twelve months from the date of issuance of these condensed consolidated financial statements. However, COPIKTRA is the Company's only approved product and the Company's business currently depends heavily on its successful commercialization. Successful commercialization of an approved product is an expensive and uncertain process. These uncertainties and risk factors raise substantial doubt regarding the Company's ability to continue as a going concern for a period of one year after the date that the financial statements are issued. Certain elements of the Company's operating plan to alleviate the conditions that raise substantial doubt are outside of the Company's control and cannot be included in management's evaluation under the requirements of Accounting Standards Codification (ASC) 205-40, *Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern*. Accordingly, the Company has concluded that substantial doubt exists about the Company's ability to continue as a going concern for a period of at least twelve months from the date of the issuance of these condensed consolidated financial statements.



## 2. Summary of significant accounting policies

### Basis of Presentation

The accompanying unaudited condensed consolidated financial statements of the Company have been prepared in accordance with generally accepted accounting principles in the United States (GAAP) for interim financial reporting and as required by Regulation S-X, Rule 10-01 under the assumption that the Company will continue as a going concern for the next twelve months. Accordingly, they do not include all of the information and footnotes required by GAAP for complete financial statements, or any adjustments that might result from the uncertainty related to the Company's ability to continue as a going concern. In the opinion of management, all adjustments (including those which are normal and recurring) considered necessary for a fair presentation of the interim financial information have been included. When preparing financial statements in conformity with GAAP, the Company must make estimates and assumptions that affect the reported amounts and related disclosures at the date of the financial statements. Actual results could differ from those estimates. Additionally, operating results for the three and six months ended June 30, 2019 are not necessarily indicative of the results that may be expected for any other interim period or for the year ending December 31, 2019. For further information, refer to the financial statements and footnotes included in the Company's Annual Report on Form 10-K for the year ended December 31, 2018, as filed with the Securities and Exchange Commission (SEC) on March 12, 2019.

### Significant Accounting Policies

The significant accounting policies identified in the Company's Annual Report on Form 10-K for the year ended December 31, 2018 that require the Company to make estimates and assumptions include accrued research and development expenses, stock-based compensation, revenue recognition, collaborative arrangements, accounts receivable, inventory and intangible assets. During the six months ended June 30, 2019, there were no material changes to the significant accounting policies, except for the adoption of Accounting Standards Codification (ASC) 842, *Leases*, issued by the Financial Accounting Standards Board (the FASB), which is detailed below.

#### *Leases*

Effective January 1, 2019, the Company adopted ASC 842. This standard requires lessees to recognize in the statement of financial position a liability to make lease payments and a right-of-use asset representing its right to use the underlying asset for the lease term for both finance and operating leases.

At the inception of an arrangement, the Company determines whether the arrangement is or contains a lease based on the unique facts and circumstances within the arrangement. A lease is identified where an arrangement conveys the right to control the use of identified property, plant, and equipment for a period of time in exchange for consideration. Leases which are identified within the scope of ASC 842 and which have a term greater than one year are recognized on the Company's condensed consolidated balance sheets as right-of-use assets, lease liabilities and, if applicable, long-term lease liabilities. The Company has elected not to recognize leases with terms of one year or less on its condensed consolidated balance sheets. Operating lease liabilities and their corresponding right-of-use assets are recorded based on the present value of lease payments over the expected remaining lease term. However, certain adjustments to the right-of-use asset may be required for items such as initial direct costs paid or incentives received. The interest rate implicit in lease contracts is typically not readily determinable. As a result, the Company utilizes its incremental borrowing rates to calculate the present value of lease payments. Incremental borrowing rates are the rates the Company incurs to borrow on a collateralized basis over a similar term an amount equal to the lease payments in a similar economic environment.

In accordance with ASC 842, components of a lease are split into three categories: lease components (e.g., land, building, etc.), non-lease components (e.g., common area maintenance, maintenance, consumables, etc.), and non-components (e.g., property taxes, insurance, etc.). The fixed and in-substance fixed contract consideration (including any related to non-components) must be allocated based on fair values to the lease components and non-lease components. Although separation of lease and non-lease components is required, certain practical expedients are available. Entities may elect the practical expedient to not separate lease and non-lease components. Rather, they would account for each lease component and the related non-lease component together as a single component. The Company has elected to

account for the lease and non-lease components of each of its operating leases as a single lease component and allocate all of the contract consideration to the lease component only. The lease component results in an operating right-of-use asset being recorded on the condensed consolidated balance sheets and amortized on a straight-line basis as lease expense.

### **Revenue Recognition**

The Company recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the Company expects to receive in exchange for those goods or services in accordance with ASC 606 *Revenue from Contracts with Customers*. To determine revenue recognition for contracts with its customers, the Company performs the following five step assessment: (i) identify the contract(s) with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) the entity satisfies a performance obligation. The Company only applies the five-step model to contracts when it is probable that the entity will collect the consideration it is entitled to in exchange for the goods or services it transfers to the customer. At contract inception and once the contract is determined to be within the scope of ASC 606, the Company assesses the goods or services promised within each contract, determines which goods and services are performance obligations, and assesses whether each promised good or service is distinct. The Company then recognizes as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied.

*Product Revenue, Net* – The Company sells COPIKTRA to a limited number of specialty pharmacies and specialty distributors in the United States. These customers subsequently resell COPIKTRA either directly to patients or to community hospitals or oncology clinics with in-office dispensaries who in turn distribute COPIKTRA to patients. In addition to distribution agreements with customers, the Company also enters into arrangements with (1) certain government agencies and various private organizations (Third-Party Payers), which may provide for chargebacks or discounts with respect to the purchase of COPIKTRA, and (2) Medicare and Medicaid, which may provide for certain rebates with respect to the purchase of COPIKTRA.

The Company recognizes revenue on sales of COPIKTRA when a customer obtains control of the product, which occurs at a point in time (typically upon delivery). Product revenues are recorded at the wholesale acquisition costs, net of applicable reserves for variable consideration. Components of variable consideration include trade discounts and allowances, Third-Party Payer chargebacks and discounts, government rebates, other incentives, such as voluntary co-pay assistance, product returns, and other allowances that are offered within contracts between the Company and customers, payors, and other indirect customers relating to the Company's sale of COPIKTRA. These reserves, as detailed below, are based on the amounts earned, or to be claimed on the related sales, and are classified as reductions of accounts receivable or a current liability. These estimates take into consideration a range of possible outcomes based upon relevant factors such as customer contract terms, information received from third parties regarding the anticipated payor mix for COPIKTRA, known market events and trends, industry data, and forecasted customer buying and payment patterns. Overall, these reserves reflect the Company's best estimates of the amount of consideration to which it is entitled with respect to sales made.

The amount of variable consideration which is included in the transaction price may be constrained and is included in the net sales price only to the extent that it is probable that a significant reversal in the amount of the cumulative revenue recognized under contracts will not occur in a future period. The Company's analyses contemplate the application of the constraint in accordance with ASC 606. For the three and six months ended June 30, 2019, the Company determined a material reversal of revenue would not occur in a future period for the estimates detailed below and, therefore, the transaction price was not reduced further. Actual amounts of consideration ultimately received may differ from the Company's estimates. If actual results in the future vary from the Company's estimates, the Company will adjust these estimates, which would affect net product revenue and earnings in the period such variances become known.

**Trade Discounts and Allowances:** The Company generally provides customers with invoice discounts on sales of COPIKTRA for prompt payment, which are explicitly stated in the Company's contracts and are recorded as a

reduction of revenue in the period the related product revenue is recognized. In addition, the Company compensates its specialty distributor customers for sales order management, data, and distribution services. The Company has determined such services are not distinct from the Company's sale of COPIKTRA to the specialty distributor customers and, therefore, these payments have also been recorded as a reduction of revenue within the condensed consolidated statements of operations and comprehensive loss for the three and six months ended June 30, 2019.

**Third-Party Payer Chargebacks, Discounts and Fees:** The Company executes contracts with Third-Party Payers which allow for eligible purchases of COPIKTRA at prices lower than the wholesale acquisition cost charged to customers who directly purchase the product from the Company. In some cases, customers charge the Company for the difference between what they pay for COPIKTRA and the ultimate selling price to the Third-Party Payers. These reserves are established in the same period that the related revenue is recognized, resulting in a reduction of product revenue and accounts receivable, net. Chargeback amounts are generally determined at the time of resale to the qualified Third-Party Payer by customers, and the Company generally issues credits for such amounts within a few weeks of the customer's notification to the Company of the resale. Reserves for chargebacks consist of credits that the Company expects to issue for units that remain in the distribution channel inventories at the end of each reporting period that the Company expects will be sold to Third-Party Payers, and chargebacks that customers have claimed, but for which the Company has not yet issued a credit. In addition, the Company compensates certain Third-Party Payers for administrative services, such as account management and data reporting. These administrative service fees have also been recorded as a reduction of product revenue within the condensed consolidated statements of operations and comprehensive loss for the three and six months ended June 30, 2019.

**Government Rebates:** The Company is subject to discount obligations under state Medicaid programs and Medicare. These reserves are recorded in the same period the related revenue is recognized, resulting in a reduction of product revenue and the establishment of a current liability which is included in accrued expenses on the condensed consolidated balance sheets. For Medicare, the Company also estimates the number of patients in the prescription drug coverage gap for whom the Company will owe an additional liability under the Medicare Part D program. The Company's liability for these rebates consists of invoices received for claims from prior quarters that have not been paid or for which an invoice has not yet been received, estimates of claims for the current quarter, and estimated future claims that will be made for product that has been recognized as revenue, but which remains in the distribution channel inventories at the end of each reporting period.

**Other Incentives:** Other incentives which the Company offers include voluntary co-pay assistance programs, which are intended to provide financial assistance to qualified commercially-insured patients with prescription drug co-payments required by payors. The calculation of the accrual for co-pay assistance is based on an estimate of claims and the cost per claim that the Company expects to receive for product that has been recognized as revenue but remains in the distribution channel inventories at the end of each reporting period. The adjustments are recorded in the same period the related revenue is recognized, resulting in a reduction of product revenue and the establishment of a current liability which is included as a component of accrued expenses on the condensed consolidated balance sheets.

**Product Returns:** Consistent with industry practice, the Company generally offers customers a limited right of return for product that has been purchased from the Company. The Company estimates the amount of its product sales that may be returned by its customers and records this estimate as a reduction of revenue in the period the related product revenue is recognized. The Company estimates product return liabilities using available industry data and its own sales information, including its visibility into the inventory remaining in the distribution channel.

Subject to certain limitations, the Company's return policy allows for eligible returns of COPIKTRA for credit under the following circumstances:

- Receipt of damaged product;
- Shipment errors that were a result of an error by the Company;
- Expired product that is returned during the period beginning three months prior to the product's expiration and ending six months after the expiration date;
- Product subject to a recall; and
- Product that the Company, at its sole discretion, has specified can be returned for credit.

The Company has not received any returns to date.

If taxes should be collected from customers relating to product sales and remitted to governmental authorities, they will be excluded from product revenue. The Company expenses incremental costs of obtaining a contract when incurred, if the expected amortization period of the asset that the Company would have recognized is one year or less. However, no such costs were incurred during the three and six months ended June 30, 2019.

*Exclusive Licenses of Intellectual Property* - The Company may enter into collaboration and licensing arrangements for research and development, manufacturing, and commercialization activities with collaboration partners for the development and commercialization of its product candidates, which have components within the scope of ASC 606. The arrangements generally contain multiple elements or deliverables, which may include (i) licenses, or options to obtain licenses, to the Company's intellectual property, (ii) research and development activities performed for the collaboration partner, (iii) participation on joint steering committees, and (iv) the manufacturing of commercial, clinical or preclinical material. Payments pursuant to these arrangements typically include non-refundable, upfront payments, milestone payments upon the achievement of significant development events, research and development reimbursements, sales milestones, and royalties on product sales. The amount of variable consideration is constrained until it is probable that the revenue is not at a significant risk of reversal in a future period. The contracts into which the Company enters generally do not include significant financing components.

In determining the appropriate amount of revenue to be recognized as it fulfills its obligations under each of its collaboration and license agreements, the Company performs the following steps: (i) identification of the promised goods or services in the contract within the scope of ASC 606; (ii) determination of whether the promised goods or services are performance obligations including whether they are distinct in the context of the contract; (iii) measurement of the transaction price, including the constraint on variable consideration; (iv) allocation of the transaction price to the performance obligations; and (v) recognition of revenue when (or as) the Company satisfies each performance obligation. As part of the accounting for these arrangements, the Company must use significant judgment to determine: a) the number of performance obligations based on the determination under step (ii) above; b) the transaction price under step (iii) above; c) the stand-alone selling price for each performance obligation identified in the contract for the allocation of transaction price in step (iv) above; and d) the measure of progress in step (v) above. The Company uses judgment to determine whether milestones or other variable consideration, except for royalties, should be included in the transaction price as described further below.

If a license to the Company's intellectual property is determined to be distinct from the other promises or performance obligations identified in the arrangement, the Company recognizes revenue from non-refundable, upfront fees allocated to the license when the license is transferred to the customer and the customer is able to use and benefit from the license. In assessing whether a promise or performance obligation is distinct from the other elements, the Company considers factors such as the research, development, manufacturing and commercialization capabilities of the collaboration partner and the availability of its associated expertise in the general marketplace. In addition, the Company considers whether the collaboration partner can benefit from a promise for its intended purpose without the receipt of the remaining elements, whether the value of the promise is dependent on the unsatisfied promise, whether there are other vendors that could provide the remaining promise, and whether it is separately identifiable from the remaining promise. For licenses that are combined with other promises, the Company utilizes judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time and, if over time, the appropriate method of measuring progress for purposes of recognizing revenue. The Company evaluates the measure of progress of each reporting period and, if necessary, adjusts the measure of performance and related revenue recognition. The measure of progress, and thereby periods over which revenue should be recognized, is subject to estimates by management and may change over the course of the arrangement. Such a change could have a material impact on the amount of revenue the Company records in future periods.

**Customer Options:** If an arrangement is determined to contain customer options that allow the customer to acquire additional goods or services such as research and development services or manufacturing services, the goods and services underlying the customer options are not considered to be performance obligations at the inception of the arrangement; rather, such goods and services are contingent on exercise of the option, and the associated option fees are

not included in the transaction price. The Company evaluates customer options for material rights or options to acquire additional goods or services for free or at a discount. If a customer option is determined to represent a material right, the material right is recognized as a separate performance obligation at the outset of the arrangement. The Company allocates the transaction price to material rights based on the relative standalone selling price, which is determined based on the identified discount and the estimated probability that the customer will exercise the option. Amounts allocated to a material right are not recognized as revenue until, at the earliest, the option is exercised.

**Milestone Payments:** At the inception of each arrangement that includes milestone payments, the Company evaluates whether the milestones are considered probable of being achieved and estimates the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal would not occur, the associated milestone value is included in the transaction price. Milestone payments that are not within the control of the Company or the licensee, such as regulatory approvals, are not considered probable of being achieved until those approvals are received. The Company evaluates factors such as the scientific, clinical, regulatory, commercial, and other risks that must be overcome to achieve the respective milestone in making this assessment. There is considerable judgment involved in determining whether it is probable that a significant revenue reversal would not occur. At the end of each subsequent reporting period, the Company reevaluates the probability of achievement of all milestones subject to constraint and, if necessary, adjusts its estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect revenues and earnings in the period of adjustment.

**Royalties:** For arrangements that include sales-based royalties, including milestone payments based on a level of sales, and the license is deemed to be the predominant item to which the royalties relate, the Company recognizes revenue at the later of (i) when the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). To date, the Company has not recognized any royalty revenue resulting from any of its licensing arrangements.

**Collaborative Arrangements:** Contracts are considered to be collaborative arrangements when they satisfy the following criteria defined in ASC 808, *Collaborative Arrangements*: (i) the parties to the contract must actively participate in the joint operating activity and (ii) the joint operating activity must expose the parties to the possibility of significant risk and rewards, based on whether or not the activity is successful. Payments received from or made to a partner that are the result of a collaborative relationship with a partner, instead of a customer relationship, such as co-development activities, are recorded as a reduction or increase to research and development expense, respectively.

#### **Concentrations of credit risk and off-balance sheet risk**

Cash, cash equivalents, short-term investments and trade accounts receivable are financial instruments that potentially subject the Company to concentrations of credit risk. The Company mitigates this risk by maintaining its cash and cash equivalents and investments with high quality, accredited financial institutions. The management of the Company's investments is not discretionary on the part of these financial institutions. As of June 30, 2019, the Company's cash, cash equivalents and short-term investments were deposited at two financial institutions and it has no significant off-balance sheet concentrations of credit risk, such as foreign currency exchange contracts, option contracts or other hedging arrangements.

As of June 30, 2019, there were two customers that cumulatively made up more than 60% of the Company's trade accounts receivable balance. The Company assesses the creditworthiness of all its customers and sets and reassesses customer credit limits to ensure collectability of any trade accounts receivable balances are assured.

For the quarter ended June 30, 2019, there were five customers who each individually accounted for greater than 10% of the Company's total revenues.

### **Recently Issued Accounting Standards Updates**

In November 2018, the Financial Accounting Standards Board (FASB) issued Accounting Standard Update (ASU) 2018-18, *Collaborative Arrangements (Topic 808): Clarifying the Interaction between Topic 808 and Topic 606*, which makes targeted improvements for collaborative arrangements to clarify that certain transactions between collaborative arrangement participants should be accounted for as revenue under Topic 606 when the collaborative arrangement participant is a customer in the context of a unit of account, adds unit of account guidance in Topic 808 to align with guidance in Topic 606, and clarifies presentation of certain revenues with a collaborative arrangement participant which are not directly related to a third party. ASU 2018-18 is effective for annual and interim periods beginning after December 15, 2019, with early adoption permitted. The Company has not elected to early adopt this standard and is currently evaluating the impact the adoption of the standard will have on its condensed consolidated financial statements and related disclosures.

In August 2018, the Financial Accounting Standards Board (FASB) issued Accounting Standard Update (ASU) 2018-15, *Intangibles-Goodwill and Other-Internal Use Software: Customer's Accounting for Implementation Costs Incurred in a Cloud Computing Arrangement that is a Service Contract*, which aligns the requirements for capitalizing implementation costs incurred in a hosting arrangement that is a service contract with the requirements for capitalizing implementation costs incurred to develop or obtain internal-use software. ASU 2018-15 is effective for annual and interim periods beginning after December 15, 2019, with early adoption permitted. The Company has not elected to early adopt this standard and is currently evaluating the impact the adoption of the standard will have on its condensed consolidated financial statements and related disclosures.

In August 2018, the FASB issued ASU 2018-13, *Fair Value Measurement (Topic 820): Disclosure Framework—Changes to the Disclosure Requirements for Fair Value Measurement*, which eliminates certain disclosure requirements for fair value measurements for all entities, requires public entities to disclose certain new information and modifies some disclosure requirements. ASU 2018-13 is effective for all entities for annual and interim periods beginning after December 15, 2019. An entity is permitted to early adopt either the entire standard or only the provisions that eliminate or modify requirements. The Company has not elected to early adopt this standard and is currently evaluating the impact the adoption of the standard will have on its condensed consolidated financial statements and related disclosures.

In June 2016, the FASB issued ASU No. 2016-13, *Measurement of Credit Losses on Financial Instruments* (“ASU 2016-13”). ASU 2016-13 will replace the incurred loss impairment methodology under current GAAP with a methodology that reflects expected credit losses and requires consideration of a broader range of reasonable and supportable information to inform credit loss estimates. The standard will be effective for annual and interim periods beginning after December 15, 2019, with early adoption permitted. The Company has not elected to early adopt this standard and is currently evaluating the impact the adoption of the standard will have on its condensed consolidated financial statements and related disclosures.

### **Recently Adopted Accounting Standards Updates**

In June 2018, the FASB issued ASU 2018-07, *Compensation – Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting*, which expands the scope of Topic 718 to include all share-based payment transactions for acquiring goods and services to be used or consumed in its own operations by issuing share-based payment awards. ASU 2018-07 also clarifies that Topic 718 does not apply to share-based payments used to effectively provide (1) financing to the issuer or (2) awards granted in conjunction with selling goods or services to customers as part of a contract and services from nonemployees. ASU 2018-07 specifies that Topic 718 applies to all share-based payment transactions accounted for under ASC 606. ASU 2018-07 was effective for annual and interim periods beginning after December 15, 2018, with early adoption permitted, but no earlier than the date on which ASC 606 is adopted. The Company adopted this standard prospectively effective January 1, 2019. The adoption of this ASU did not have an effect on the Company's condensed consolidated financial statements or related disclosures.

In February 2016, the FASB issued ASU 2016-02, *Leases (Topic 842)*, which supersedes the guidance under FASB Accounting Standards Codification (ASC) Topic 840, *Leases*, resulting in the creation of FASB ASC Topic 842, *Leases (ASC 842)*. ASU 2016-02 requires lessees to recognize in the statement of financial position a liability to make lease payments and a right-of-use asset representing its right to use the underlying asset for the lease term for both finance and operating leases. The guidance also eliminates the current real estate-specific provisions for all entities. In July 2018, the FASB issued ASU 2018-11, *Leases (Topic 842): Targeted Improvements*, which provides entities with relief from the costs of implementing certain aspects of the new leasing standard, ASU 2016-02. Under the amendments in ASU 2018-11, entities may elect not to restate the comparative periods presented when transitioning to ASC 842 (optional transition method) and lessors may elect not to separate lease and non-lease components when certain conditions are met (lessor relief practical expedient). The optional transition method applies to entities that have not yet adopted ASU 2016-02, which is effective for fiscal years, and interim periods within those years, beginning after December 15, 2018, with early adoption permitted.

The Company adopted this standard using the optional transition method effective January 1, 2019. Upon adoption of this standard, the Company recognized a lease liability and a corresponding right-of use asset of \$4.0 million and \$3.4 million, respectively, and derecognized a deferred rent liability and a corresponding lease incentive obligation of \$0.4 million and \$0.2 million, respectively. The Company did not record any cumulative effect adjustment to accumulated deficit as a result of adopting this standard. The Company also elected to adopt the practical expedients upon transition, which permit companies to not reassess lease identification, classification, and initial direct costs under ASU 2016-02 for leases that commenced prior to the effective date.

### 3. Cash, cash equivalents and restricted cash

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

	<u>June 30, 2019</u>	<u>December 31, 2018</u>
Cash and cash equivalents	\$ 113,080	\$ 129,867
Restricted cash	741	741
<b>Total cash, cash equivalents and restricted cash</b>	<b>\$ 113,821</b>	<b>\$ 130,608</b>

Amounts included in restricted cash as of June 30, 2019 and December 31, 2018 represent cash received pursuant to a Research Funding Agreement with Leukemia & Lymphoma Society, Inc. (LLS), which cash is restricted for future expenditures for specific R&D studies in the amount of approximately \$0.5 million, respectively. Restricted cash also includes cash held to collateralize outstanding letters of credit provided as a security deposit for the Company's office space located in Needham, Massachusetts, in the amount of approximately \$0.2 million, respectively. Restricted cash related to the LLS Research Funding Agreement is included in prepaid and other current assets, while restricted cash for letters of credit are included in other assets on the consolidated balance sheets.

### 4. Fair value of financial instruments

The Company determines the fair value of its financial instruments based upon the fair value hierarchy, which prioritizes valuation inputs based on the observable nature of those inputs. The fair value hierarchy applies only to the valuation inputs used in determining the reported fair value of the investments and is not a measure of the investment credit quality. The hierarchy defines three levels of valuation inputs:

Level 1 inputs	Quoted prices in active markets for identical assets or liabilities that the Company can access at the measurement date.
Level 2 inputs	Inputs other than quoted prices included within Level 1 that are observable for the asset or liability, either directly or indirectly.
Level 3 inputs	Unobservable inputs that reflect the Company's own assumptions about the assumptions market participants would use in pricing the asset or liability.

**Items Measured at Fair Value on a Recurring Basis**

The following table presents information about the Company's financial instruments that are measured at fair value on a recurring basis (in thousands):

Description	June 30, 2019			
	Total	Level 1	Level 2	Level 3
<b>Financial assets</b>				
Cash equivalents	\$ 111,174	\$ 111,174	\$ —	\$ —
Short-term investments	74,173	—	74,173	—
<b>Total financial assets</b>	<b>\$ 185,347</b>	<b>\$ 111,174</b>	<b>\$ 74,173</b>	<b>\$ —</b>

Description	December 31, 2018			
	Total	Level 1	Level 2	Level 3
<b>Financial assets</b>				
Cash equivalents	\$ 127,689	\$ 60,092	\$ 67,597	\$ —
Short-term investments	119,786	—	119,786	—
<b>Total financial assets</b>	<b>\$ 247,475</b>	<b>\$ 60,092</b>	<b>\$ 187,383</b>	<b>\$ —</b>

The Company's cash equivalents and short-term investments consist of U.S. Government money market funds, corporate bonds and commercial paper of publicly traded companies. The investments and cash equivalents have been initially valued at the transaction price and subsequently valued, at the end of each reporting period, utilizing third party pricing services or other market observable data. The pricing services utilize industry standard valuation models, including both income and market-based approaches and observable market inputs to determine value. These observable market inputs include reportable trades, benchmark yields, credit spreads, broker/dealer quotes, bids, offers, current spot rates and other industry and economic events. The Company validates the prices provided by third party pricing services by reviewing their pricing methods and matrices, obtaining market values from other pricing sources, analyzing pricing data in certain instances and confirming that the relevant markets are active. After completing its validation procedures, the Company did not adjust or override any fair value measurements provided by the pricing services as of June 30, 2019 and December 31, 2018.

**Fair Value of Financial Instruments**

The fair value of the Company's long-term debt is determined using a discounted cash flow analysis with current applicable rates for similar instruments as of the condensed consolidated balance sheet dates. The carrying value of the Company's long-term debt, including the current portion, at June 30, 2019 and December 31, 2018 was approximately \$34.7 million and \$25.2 million, respectively. At June 30, 2019, the Company estimates that the fair value of its long-term debt, including the current portion, was approximately \$37.1 million. The fair value of the Company's long-term debt was determined using Level 3 inputs.

The fair value of the Company's 5.00% Convertible Senior Notes due 2048 (the Notes) as of June 30, 2019 was approximately \$77.6 million, which differs from the carrying value of the Notes. The fair value of the Notes was determined using Level 2 inputs.



## 5. Investments

Cash, cash equivalents, and short-term investments consist of the following (in thousands):

	June 30, 2019			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Cash and cash equivalents:				
Cash and money market accounts	\$ 113,080	\$ —	\$ —	\$ 113,080
Corporate bonds and commercial paper (due within 90 days)	—	—	—	—
<b>Total cash and cash equivalents</b>	<b>\$ 113,080</b>	<b>\$ —</b>	<b>\$ —</b>	<b>\$ 113,080</b>
Investments:				
Corporate bonds and commercial paper (due within 1 year)	\$ 74,088	\$ 86	\$ —	\$ 74,173
<b>Total investments</b>	<b>\$ 74,088</b>	<b>\$ 86</b>	<b>\$ —</b>	<b>\$ 74,173</b>
<b>Total cash, cash equivalents and investments</b>	<b>\$ 187,168</b>	<b>\$ 86</b>	<b>\$ —</b>	<b>\$ 187,253</b>

	December 31, 2018			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Cash and cash equivalents:				
Cash and money market accounts	\$ 62,270	\$ —	\$ —	\$ 62,270
Corporate bonds and commercial paper (due within 90 days)	67,590	\$ 8	\$ (1)	\$ 67,597
<b>Total cash and cash equivalents</b>	<b>\$ 129,860</b>	<b>\$ 8</b>	<b>\$ (1)</b>	<b>\$ 129,867</b>
Investments:				
Corporate bonds and commercial paper (due within 1 year)	\$ 119,666	\$ 132	\$ (12)	\$ 119,786
<b>Total investments</b>	<b>\$ 119,666</b>	<b>\$ 132</b>	<b>\$ (12)</b>	<b>\$ 119,786</b>
<b>Total cash, cash equivalents and investments</b>	<b>\$ 249,526</b>	<b>\$ 140</b>	<b>\$ (13)</b>	<b>\$ 249,653</b>

There were no realized gains or losses on investments for the three and six months ended June 30, 2019 or 2018, respectively. There were zero and fourteen investments in an unrealized loss position as of June 30, 2019 and December 31, 2018, respectively. None of these investments had been in an unrealized loss position for more than 12 months as of June 30, 2019 and December 31, 2018, respectively. The fair value of these securities as of June 30, 2019 and December 31, 2018 was \$0 and \$46.9 million, respectively, and the aggregate unrealized loss was immaterial. The Company considered the decline in the market value for these securities to be primarily attributable to current economic conditions. As it was not more likely than not that the Company would be required to sell these securities before the recovery of their amortized cost basis, which may be at maturity, the Company did not consider these investments to be other-than-temporarily impaired as of June 30, 2019 and December 31, 2018, respectively.

## 6. Inventory

During the third quarter of 2018, the Company began capitalizing inventory costs for COPIKTRA manufactured in preparation for its launch in the United States based on its evaluation of, among other factors, the status of the COPIKTRA New Drug Application (NDA) in the United States and the ability of its third-party suppliers to successfully manufacture commercial quantities of COPIKTRA, which provided the Company with reasonable assurance that the net realizable value of the inventory would be recoverable.

Inventory consists of the following (in thousands):

	<u>June 30, 2019</u>	<u>December 31, 2018</u>
Raw materials	\$ —	\$ —
Work in process	119	63
Finished goods	175	264
<b>Total inventories</b>	<b>\$ 294</b>	<b>\$ 327</b>

Costs incurred prior to the quarter-ended September 30, 2018 to manufacture COPIKTRA were expensed as operating expenses as incurred.

## 7. Intangible assets

The Company's intangible assets consist of the following (in thousands):

	<u>June 30, 2019</u>	<u>Estimated useful life</u>
Acquired and in-licensed rights	\$ 22,000	14 years
Less: accumulated amortization	(1,207)	
<b>Total intangible assets, net</b>	<b>\$ 20,793</b>	

Acquired and in-licensed rights as of June 30, 2019, consist of a \$22.0 million milestone payment which became payable upon the FDA marketing approval on September 24, 2018 pursuant to the amended and restated license agreement with Infinity Pharmaceuticals, Inc. (Infinity). The Company made the milestone payment of \$22.0 million to Infinity in November 2018.

The Company recorded approximately \$0.4 million and \$0.8 million in amortization expense related to finite-lived intangible assets during the three and six months ended June 30, 2019 using the straight-line methodology. Estimated future amortization expense for finite-lived intangible assets as of June 30, 2019 is approximately \$0.8 million for the remainder of 2019 and approximately \$1.6 million per year thereafter.

## 8. Accrued expenses

Accrued expenses consist of the following (in thousands):

	<u>June 30, 2019</u>	<u>December 31, 2018</u>
Compensation and related benefits	6,689	8,749
Contract research organization costs	6,516	6,682
Commercialization costs	2,298	1,979
Interest	1,534	1,786
Consulting fees	1,267	494
Professional fees	661	482
Other	407	936
<b>Total accrued expenses</b>	<b>\$ 19,372</b>	<b>\$ 21,108</b>

## 9. Product revenue reserves and allowances

As of June 30, 2019, the Company's sole source of product revenue has been from sales of COPIKTRA in the United States, which it began shipping to customers on September 25, 2018. The following table summarizes activity in each of the product revenue allowance and reserve categories for the three and six months ended June 30, 2019 (in thousands):

	Trade discounts and allowances	Third-Party Payer chargebacks, discounts and fees	Government rebates and other incentives	Returns	Total
Beginning balance at December 31, 2018	\$ 29	\$ 88	\$ 157	\$ 2	\$ 276
Provision related to sales in the current year	75	136	177	2	390
Adjustments related to prior period sales	—	—	(32)	—	(32)
Credits and payments made	(61)	(120)	(20)	—	(201)
<b>Ending Balance at March 31, 2019</b>	<b>\$ 43</b>	<b>\$ 104</b>	<b>\$ 282</b>	<b>\$ 4</b>	<b>\$ 433</b>
Provision related to sales in the current year	111	280	76	4	471
Adjustments related to prior period sales	—	—	(44)	—	(44)
Credits and payments made	(102)	(231)	(115)	—	(448)
<b>Ending balance at June 30, 2019</b>	<b>\$ 52</b>	<b>\$ 153</b>	<b>\$ 199</b>	<b>\$ 8</b>	<b>\$ 412</b>

Trade discounts and Third-Party Payer chargebacks and discounts are recorded as a reduction to accounts receivable, net on the condensed consolidated balance sheets. Trade allowances and Third-Party Payer fees, government rebates, other incentives and returns are recorded as a component of accrued expenses on the condensed consolidated balance sheets.

## 10. Leases

On April 15, 2014, the Company entered into a lease agreement for approximately 15,197 square feet of office and laboratory space in Needham, Massachusetts. Effective February 15, 2018, the Company amended its lease agreement to relocate within the facility to another location consisting of 27,810 square feet of office space (the Amended Lease Agreement). The Amended Lease Agreement extends the expiration date of the lease from September 2019 through May 2025. Pursuant to the Amended Lease Agreement, the initial annual base rent amount is approximately \$0.7 million, which increases during the lease term to \$1.1 million for the last twelve-month period.

The Company has accounted for its Needham, Massachusetts office space as an operating lease. The Company's lease contains an option to renew and extend the lease terms and an option to terminate the lease prior to the expiration date. The Company has not included the lease extension or the termination options within the right-of-use asset and lease liability on the condensed consolidated balance sheets as neither option is reasonably certain to be exercised. The Company's lease includes variable non-lease components (e.g., common area maintenance, maintenance, consumables, etc.) that are not included in the right-of-use asset and lease liability and are reflected as an expense in the period incurred. The Company does not have any other operating or finance leases.

In calculating the present value of future lease payments, the Company has elected to utilize its incremental borrowing rate based on the remaining lease term at the date of adoption of ASC 842. The Company has elected to account for lease components and associated non-lease components as a single lease component and has allocated all of the contract consideration to the lease components only. This will potentially result in the initial and subsequent measurement of the balances of the right-of-use asset and lease liability for leases being greater than if the policy election was not applied.

As of June 30, 2019, a right-of-use asset of \$3.2 million and lease liability of \$4.0 million are reflected on the condensed consolidated balance sheets. The elements of lease expense were as follows (dollar amounts in thousands):

	Three months ended June 30, 2019	Six months ended June 30, 2019
<b>Lease Expense</b>		
Operating lease expense	\$ 222	\$ 444
<b>Total Lease Expense</b>	<b>\$ 222</b>	<b>\$ 444</b>
<b>Other Information - Operating Leases</b>		
Operating cash flows paid for amounts included in measurement of lease liabilities	\$ 166	\$ 331
		<b>June 30, 2019</b>
<b>Other Balance Sheet Information - Operating Leases</b>		
Weighted average remaining lease term (in years)		5.9
Weighted average discount rate		14.60%
<b>Maturity Analysis</b>		
Remainder of 2019	\$	385
2020		971
2021		1,020
2022		1,041
2023		1,062
Thereafter		1,538
<b>Total</b>	<b>\$</b>	<b>6,017</b>
Less: Present value discount		(2,029)
<b>Lease Liability</b>	<b>\$</b>	<b>3,988</b>

The Company adopted ASU 2016-02 effective January 1, 2019 using the optional transition method permitted under ASU 2018-11. Accordingly, periods presented prior to January 1, 2019 were not restated to reflect the accounting principles adopted under ASU 2016-02. Prior to adoption, the Company recorded rent expense from its Needham office on a straight-line basis over the term of the lease with the deferred rent obligation included in accrued expenses (current portion) and other liabilities (noncurrent portion) in the condensed consolidated balance sheet as of December 31, 2018. The Company amortized any leasehold improvements over the lesser of the useful life of those improvements or the life of the lease. For the three and six months ended June 30, 2018, the Company recorded rent expense of \$0.2 and \$0.3 million.

At December 31, 2018, future minimum lease payments under non-cancelable leases under ASC 840 were as follows (in thousands):

2019	\$ 716
2020	971
2021	1,020
2022	1,041
2023	1,062
Thereafter	1,538
<b>Total</b>	<b>\$ 6,348</b>

## 11. Long-term debt

On March 21, 2017, the Company entered into a term loan facility of up to \$25.0 million with Hercules Capital, Inc. (Hercules). The term loan facility is governed by a loan and security agreement, dated March 21, 2017 (the Original Loan Agreement), which was amended on January 4, 2018, March 6, 2018 and October 11, 2018 (the Amended Loan Agreement) to increase the total borrowing limit under the Original Loan Agreement from up to \$25.0 million to up to \$50.0 million (the Amended Term Loan), pursuant to certain conditions of funding. On April 23, 2019 (the Amendment Date), the Company entered into the Fourth Amendment (the Amendment) to the Loan and Security Agreement with Hercules. The Amendment amends the Amended Loan Agreement (together with the Amendment, the 2019 Term Loan Agreement).

Per the terms of the Amendment, the Company may borrow up to an aggregate of \$75.0 million, of which \$35.0 million was outstanding immediately as of the Amendment Date (2019 Term A Loan) as a result of the existing outstanding principal of term loans of \$25.0 million under the Amended Loan Agreement being converted into the 2019 Term A Loan, and an additional \$10.0 million being drawn on the Amendment Date. The remaining \$40.0 million of borrowing capacity may be drawn in multiple tranches comprised of (i) a term loan in an amount of up to \$15.0 million upon the Company generating cumulative net product revenues (as defined in the 2019 Term Loan Agreement) of either (a) \$37.5 million on or before April 30, 2020 or (b) \$50.0 million on or before June 30, 2020 (2019 Term B Loan), and (ii) a term loan in an amount of up to \$25.0 million available through December 31, 2021, subject to Hercules' approval and certain other conditions specified in the 2019 Term Loan Agreement (the 2019 Term C Loan, and together with the 2019 Term A Loan and 2019 Term B Loan, the 2019 Term Loan).

The 2019 Term Loan will mature on December 1, 2022 (2019 Term Loan Maturity Date). Each advance accrues interest at a floating per annum rate equal to the greater of (a) 9.75% or (b) the lesser of (i) 12.00% and (ii) the sum of (x) 9.75% plus (y) (A) the prime rate minus (B) 5.50%. In addition, the Company is required to make a final payment equal to 5.25% of the aggregate original principal balance of all advances upon the earliest to occur (a) 2019 Term Loan Maturity Date, (b) prepayment of the debt or (c) the date the 2019 Term Loan becomes due and payable. The 2019 Term Loan provides for interest-only payments until April 1, 2021, which may be extended to December 1, 2021 pursuant to the Company generating \$40.0 million in net product revenue on a trailing six-month basis on or prior to December 31, 2020 provided that no event of default has occurred. Thereafter, amortization payments will be payable monthly in equal installments of principal and interest (subject to recalculation upon a change in prime rates). As such, all outstanding balances related to the Amended Term Loan have been classified as long-term debt on the condensed consolidated Balance Sheet as of June 30, 2019.

The 2019 Term Loan is secured by a lien on substantially all of the assets of the Company, other than intellectual property and contains customary covenants and representations, including a liquidity covenant, minimum net revenue covenant, financial reporting covenant and limitations on dividends, indebtedness, collateral, investments, distributions, transfers, mergers or acquisitions, taxes, corporate changes, deposit accounts, and subsidiaries.

On the Amendment Date, the Company was required to pay any outstanding accrued interest as well as the final payment fee equal to 4.5% on the outstanding principal balance of the Amended Term Loan, or \$1.1 million on the existing term loans. No prepayment charges were due as a result of executing the Amendment or conversion of the existing term loans into 2019 Term A Loans.

As of June 30, 2019, the Company has assessed all terms and features of the 2019 Term Loan Agreement in order to identify any potential embedded features that would require bifurcation or any beneficial conversion features. As part of this analysis, the Company assessed the economic characteristics and risks of the 2019 Term Loan Agreement, including put and call features. The Company determined that all features of the 2019 Term Loan Agreement were clearly and closely associated with a debt host and did not require bifurcation as a derivative liability, or the fair value of the feature was immaterial to the Company's condensed consolidated financial statements. The Company reassesses the

features on a quarterly basis to determine if they require separate accounting. There have been no changes to the Company's original assessment through June 30, 2019.

The future principal payments under the 2019 Term Loan are as follows as of June 30, 2019 (in thousands):

2021	\$	14,234
2022		20,766
<b>Total principal payments</b>	<b>\$</b>	<b>35,000</b>

## 12. Convertible Senior Notes

On October 17, 2018, the Company closed a registered direct public offering of \$150.0 million aggregate principal amount of the Company's 5.00% Convertible Senior Notes due 2048 (the Notes). The Notes are governed by the terms of a base indenture for senior debt securities (the Base Indenture), as supplemented by the first supplemental indenture thereto (the Supplemental Indenture and together with the Base Indenture, the Indenture), each dated October 17, 2018, by and between the Company and Wilmington Trust, National Association, as trustee. The Notes are senior unsecured obligations of the Company and bear interest at a rate of 5.00% per annum, payable semi-annually in arrears on May 1 and November 1 of each year, beginning on May 1, 2019. The Notes will mature on November 1, 2048, unless earlier repurchased, redeemed or converted in accordance with their terms.

The Notes are convertible into shares of the Company's common stock, par value \$0.0001 per share, together, if applicable, with cash in lieu of any fractional share, at an initial conversion rate of 139.5771 shares of common stock per \$1,000 principal amount of the Notes, which corresponds to an initial conversion price of approximately \$7.16 per share of common stock and represents a conversion premium of approximately 15.0% above the last reported sale price of the common stock of \$6.23 per share on October 11, 2018. Upon conversion, converting noteholders will be entitled to receive accrued interest on their converted Notes. The conversion rate is subject to adjustment from time to time upon the occurrence of certain events, including, but not limited to, the issuance of stock dividends and payment of cash dividends, but will not be adjusted for any accrued and unpaid interest.

The Indenture includes customary covenants and sets forth certain events of default after which the Notes may be declared immediately due and payable and sets forth certain types of bankruptcy or insolvency events of default involving the Company or certain of its subsidiaries after which the Notes become automatically due and payable.

The Company assessed all terms and features of the Notes in order to identify any potential embedded features that would require bifurcation. As part of this analysis, the Company assessed the economic characteristics and risks of the Notes, including the conversion, put and call features. The conversion feature was initially bifurcated as an embedded derivative but subsequently qualified for a scope exception to derivative accounting upon the Company's stockholders approving an increase in the number of authorized shares of Common Stock. The Company determined that all other features of the Notes were clearly and closely associated with the debt host and did not require bifurcation as a derivative liability, or the fair value of the feature was immaterial to the Company's condensed consolidated financial statements. The Company reassesses the features on a quarterly basis to determine if they require separate accounting. There have been no changes to the Company's original assessment through June 30, 2019.

### 13. Stock-based compensation

#### Stock options

A summary of the Company's stock option activity and related information for the six months ended June 30, 2019 is as follows:

	Shares	Weighted-average exercise price per share	Weighted-average remaining contractual term (years)	Aggregate intrinsic value (in thousands)
Outstanding at December 31, 2018	12,522,867	\$ 5.42	7.8	\$ 6,909
Granted	4,978,840	\$ 2.58		
Exercised	(46,803)	\$ 1.60		
Forfeited/cancelled	(819,147)	\$ 4.31		
Outstanding at June 30, 2019	16,635,757	\$ 4.64	7.4	\$ 450
Vested at June 30, 2019	7,304,693	\$ 5.55	5.5	\$ 334
Vested and expected to vest at June 30, 2019(1)	15,922,757	\$ 4.69	7.4	\$ 450

- (1) This represents the number of vested options as of June 30, 2019, plus the number of unvested options expected to vest as of June 30, 2019.

The fair value of each stock option granted during the three months ended June 30, 2019 and 2018 was estimated on the grant date using the Black-Scholes option-pricing model using the following weighted-average assumptions:

	Six months ended June 30,	
	2019	2018
Risk-free interest rate	2.16 %	2.51 %
Volatility	86 %	81 %
Dividend yield	—	—
Expected term (years)	5.8	6.0

During the first quarter of 2018, the Company granted stock options to purchase a total of 582,500 shares of common stock to certain executives that vest only upon the achievement of specified performance conditions. The Company determined that a number of the performance conditions are considered probable of achievement as of June 30, 2018, and as a result recognized approximately \$0.2 million and \$0.5 million of stock-based compensation expense during the three and six months ended June 30, 2018 related to awards that vest upon the achievement of performance conditions. No stock-based compensation expense has been recognized during the three and six months ended June 30, 2019 related to awards that vest upon the achievement of performance conditions.

At June 30, 2019, there was \$21.4 million of total unrecognized compensation cost related to unvested stock options and the Company expects to recognize this cost over a remaining weighted-average period of approximately 4.06 years.

#### Restricted stock units (RSUs)

The Company awards RSUs to employees under its Amended and Restated 2012 Incentive Plan and its inducement award program. Each RSU entitles the holder to receive one share of the Company's common stock when the RSU vests. The RSUs generally vest in either (i) four substantially equal installments on each of the first four anniversaries of the vesting commencement date, or (ii) 100 percent on the first anniversary of the vesting commencement date, subject to the employee's continued employment with, or service to, the Company on such vesting date. Compensation expense is recognized on a straight-line basis.

A summary of RSU activity during the six months ended June 30, 2019 is as follows:

	Shares	Weighted- average grant date fair value per share
Outstanding at December 31, 2018	306,750	\$ 5.24
Granted	528,904	\$ 3.12
Vested	(36,439)	\$ 3.00
Forfeited/cancelled	(60,098)	\$ 3.94
Outstanding at June 30, 2019	739,117	\$ 3.98

At June 30, 2019, there was approximately \$2.0 million of total unrecognized compensation cost related to unvested RSUs and the Company expects to recognize this cost over a remaining weighted-average period of approximately 2.90 years.

#### **Employee stock purchase plan**

At the Special Meeting of Stockholders, held on December 18, 2018, the stockholders approved the 2018 Employee Stock Purchase Plan (2018 ESPP). On June 21, 2019, the board of directors of the Company amended and restated the 2018 ESPP, to account for certain non-material changes to the plan's administration (the Amended and Restated 2018 ESPP). The Amended and Restated 2018 ESPP provides eligible employees with the opportunity, through regular payroll deductions, to purchase shares of the Company's common stock at 85% of the lesser of the fair market value of the common stock (a) on the date the option is granted, which is the first day of the purchase period, and (b) on the exercise date, which is the last business day of the purchase period. The Amended and Restated 2018 ESPP generally allows for two six-month purchase periods per year beginning in January and July, or such other periods as determined by the compensation committee of our board of directors. The initial purchase plan period commenced in February 2019. The Company has reserved 2,000,000 shares of common stock for the administration of the Amended and Restated 2018 ESPP. The fair value of shares expected to be purchased under the Amended and Restated 2018 ESPP was calculated using the Black-Scholes model with the following assumptions:

	Six months ended June 30, 2019
Risk-free interest rate	2.46 %
Volatility	79 %
Dividend yield	—
Expected term (years)	0.4

For the three and six months ended June 30, 2019, the Company has recognized \$0.1 million and \$0.3 million of stock-based compensation under the Amended and Restated 2018 ESPP. As of June 30, 2019, there have been no purchases of the Company's common stock under the plan.

#### **14. Net loss per share**

Basic net loss per common share is calculated by dividing net loss applicable to common stockholders by the weighted-average number of common shares outstanding during the period. Diluted net loss per common share is calculated by increasing the denominator by the weighted-average number of additional shares that could have been outstanding from securities convertible into common stock, such as stock options and restricted stock units (using the "treasury stock" method), and the Notes (using the "if-converted" method), unless their effect on net loss per share is antidilutive.

The following potentially dilutive securities were excluded from the calculation of diluted net loss per share for the periods indicated because including them would have had an anti-dilutive effect:



	Three months ended June 30,		Six months ended June 30,	
	2019	2018	2019	2018
Outstanding stock options	16,635,757	11,390,340	16,635,757	11,390,340
Outstanding restricted stock units	739,117	162,125	739,117	162,125
Convertible senior notes	20,936,548	—	20,936,548	—
<b>Total potentially dilutive securities</b>	<b>38,311,422</b>	<b>11,552,465</b>	<b>38,311,422</b>	<b>11,552,465</b>

## 15. License and collaboration agreements

### *Yakult Honsha Co., Ltd. (Yakult)*

On June 5, 2018, the Company entered into a license and collaboration agreement with Yakult (the Yakult Agreement), under which the Company granted exclusive rights to Yakult to develop and commercialize products containing duvelisib in Japan for the treatment, prevention, palliation or diagnosis of all oncology indications in humans or animals. Under the terms of the Yakult Agreement, Yakult received an exclusive right to develop and commercialize products containing duvelisib in Japan under mutually agreed upon development and commercialization plans at its own cost and expense. Yakult also received certain limited manufacturing rights in the event that the Company is unable to manufacture or supply sufficient quantities of duvelisib or products containing duvelisib to Yakult during the term of the Yakult Agreement. The Company retained all rights to duvelisib outside of Japan.

Yakult paid the Company an upfront, non-refundable payment of \$10.0 million in June 2018. The Company is also entitled to receive aggregate payments of up to \$90.0 million if certain development, regulatory and commercial milestones are successfully achieved. Yakult is obligated to pay the Company a double-digit royalty on net sales of products containing duvelisib in Japan, subject to reduction in certain circumstances, and to fund certain global development costs related to worldwide clinical trials conducted by the Company in which Yakult has opted to participate on a pro-rata basis. For the three and six months ended June 30, 2019, there have been no additional milestones achieved under the Yakult Agreement.

Subsequently, on February 28, 2019, the Company entered into a supply agreement with Yakult (the Yakult Supply Agreement), under which the Company agreed to provide Yakult with drug product for clinical and commercial use in accordance with the Yakult Agreement. Under the terms of the Yakult Supply Agreement, the Company also granted to Yakult a limited manufacturing license to fill, finish, package, and label the drug product solely for clinical and commercial purposes in Japan.

Unless earlier terminated by either party, the Yakult Agreement will expire upon the fulfillment of Yakult's royalty obligations to the Company for the sale of any products containing duvelisib in Japan, which royalty obligations expire, on a product-by-product basis, upon the last to occur of (a) expiration of valid claims covering such product, (b) expiration of regulatory exclusivity for such product or (c) 10 years from first commercial sale of such product. Yakult may terminate the Yakult Agreement in its entirety at any time with 180 days' written notice. Either party may terminate the Yakult Agreement in its entirety with 60 days' written notice for the other party's material breach if such party fails to cure the breach. The Company may terminate the Yakult Agreement if (i) Yakult fails to use commercially reasonable efforts to develop and commercialize products containing duvelisib in Japan or (ii) Yakult challenges any patent licensed by the Company to Yakult under the Yakult Agreement. Either party may terminate the Yakult Agreement in its entirety upon certain insolvency events involving the other party.

### *CSPC Pharmaceutical Group Limited (CSPC)*

On September 25, 2018, the Company entered into a license and collaboration agreement with CSPC (the CSPC Agreement), under which the Company granted exclusive rights to CSPC to develop and commercialize products containing duvelisib in the People's Republic of China (China), Hong Kong, Macau and Taiwan (each, a Region and collectively, the CSPC Territory) for the treatment, prevention, palliation or diagnosis of all oncology indications in humans.

Under the terms of the CSPC Agreement, CSPC received an exclusive right to develop and commercialize products containing duvelisib in the CSPC Territory under mutually agreed upon development and commercialization plans at its own cost and expense. CSPC also received certain limited manufacturing rights in the event that the Company is unable to manufacture or supply sufficient quantities of duvelisib or products containing duvelisib to CSPC during the term of the CSPC Agreement. The Company retained all rights to duvelisib outside of the CSPC Territory.

The Company is entitled to receive aggregate payments of up to \$160.0 million if certain development, regulatory and commercial milestones are successfully achieved. CSPC is obligated to pay the Company a double-digit royalty on net sales of products containing duvelisib in the CSPC Territory, subject to reduction in certain circumstances, and to fund certain global development costs related to worldwide clinical trials conducted by the Company in which CSPC has opted to participate on a pro-rata basis. For the three months ended June 30, 2019, there have been no additional milestones achieved under the CSPC Agreement.

Unless earlier terminated by either party, the CSPC Agreement will expire upon the fulfillment of CSPC's royalty obligations to the Company for the sale of any products containing duvelisib in the CSPC Territory, which royalty obligations expire, on a product-by-product basis, upon the last to occur, in each specific Region of (a) expiration of valid claims covering such product, (b) expiration of regulatory exclusivity for such product or (c) 10 years from first commercial sale of such product. CSPC may terminate the CSPC Agreement in its entirety at any time with 180 days' written notice. Either party may terminate the CSPC Agreement in its entirety with 60 days' written notice for the other party's material breach if such party fails to cure the breach. The Company may terminate the CSPC Agreement immediately if CSPC breaches its non-compete obligations or any of its representations and warranties or covenants under the CSPC Agreement. The Company may also terminate the CSPC Agreement if (i) CSPC fails to use commercially reasonable efforts to develop and commercialize products containing duvelisib in and Region or the CSPC Territory or (ii) CSPC challenges any patent licensed by the Company to CSPC under the CSPC Agreement. Either party may terminate the CSPC Agreement in its entirety upon certain insolvency events involving the other party.

#### **16. Income taxes**

The Company did not record a federal or state income tax provision or benefit for the three and six months ended June 30, 2019 and 2018, respectively, due to the expected loss before income taxes to be incurred for the years ended December 31, 2019 and 2018, as well as the Company's continued maintenance of a full valuation allowance against its net deferred tax assets.

#### **17. Commitments and contingencies**

The Company has entered into a lease agreement for approximately 27,810 square feet of office space in Needham, Massachusetts. Please refer to Note 10 for further details regarding the minimum aggregate future lease commitments as of June 30, 2019. In conjunction with the execution of the Amended Lease Agreement, the Company has provided a security deposit in the form of a letter of credit in the amount of \$0.2 million as of June 30, 2019 and December 31, 2018. The amount is included in other assets on the condensed consolidated balance sheets as of June 30, 2019.

#### **18. Subsequent events**

The Company reviews all activity subsequent to the end of the quarter but prior to issuance of the condensed consolidated financial statements for events that could require disclosure or that could impact the carrying value of assets or liabilities as of the balance sheet date. The Company is not aware of any material subsequent events other than the following:

### ***Verastem Europe GmbH***

On July 11, 2019, Verastem Europe GmbH, a wholly-owned subsidiary of Verastem, Inc., was registered in Munich, Germany. The Company is currently pursuing regulatory approval for COPIKTRA in the European Union and is evaluating potential commercialization strategies in the EU should it be successfully in achieving such approval.

### ***Sanofi Agreement***

On July 25, 2019, the Company entered into an exclusive license agreement with Sanofi under which the Company granted exclusive rights to Sanofi to develop and commercialize products containing COPIKTRA (duvelisib) in Russia, the Commonwealth of Independent States, Turkey, the Middle East and Africa. Under the terms of the agreement, the Company will receive an upfront payment of \$5.0 million and will be eligible to receive up to an additional \$42.0 million in development and sales milestone payments, plus double-digit percentage royalties based on future net sales of COPIKTRA in the licensed territories. Sanofi will receive exclusive rights to develop and commercialize COPIKTRA, and hold the marketing authorization and product license for COPIKTRA, in the licensed territories. Sanofi will also have the right to collaborate with the Company on certain global development and clinical trial activities.

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

*You should read the following discussion and analysis of our financial condition and results of operations together with our condensed consolidated financial statements and related notes appearing elsewhere in this Quarterly Report on Form 10-Q. The following discussion contains forward-looking statements that involve risks and uncertainties. Our actual results and the timing of certain events could differ materially from those anticipated in these forward-looking statements as a result of certain factors, including those discussed below and elsewhere in this Quarterly Report on Form 10-Q and in our Annual Report on Form 10-K for our fiscal year ended December 31, 2018. Please also refer to the sections under headings "Forward-Looking Statements" and "Risk Factors" in this Quarterly Report on Form 10-Q and in our Annual Report on Form 10-K for our fiscal year ended December 31, 2018.*

### OVERVIEW

We are a biopharmaceutical company focused on developing and commercializing medicines to improve the survival and quality of life of cancer patients. Both our marketed product, COPIKTRA™ (duvelisib) capsules, and most advanced product candidate, defactinib, utilize a multi-faceted approach designed to treat certain cancers originating either in the blood or major organ systems. We are currently developing our product candidates in both preclinical and clinical studies as potential therapies for certain cancers, including leukemia, lymphoma, lung cancer, mesothelioma, ovarian cancer, colorectal cancer, head and neck cancer, and pancreatic cancer. We believe that these compounds may be beneficial as therapeutics either as single agents or when used in combination with immuno-oncology agents or other current and emerging standard of care treatments in aggressive cancers that do not adequately respond to currently available therapies.

COPIKTRA is an oral inhibitor of phosphoinositide 3-kinase (PI3K), and the first approved dual inhibitor of PI3K-delta and PI3K-gamma, two enzymes known to help support the growth and survival of malignant B-cells. PI3K signaling may lead to the proliferation of malignant B-cells and is thought to play a role in the formation and maintenance of the supportive tumor microenvironment. COPIKTRA was approved by the U.S. Food & Drug Administration (FDA) on September 24, 2018, and is now indicated for the treatment of adult patients with relapsed or refractory chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL) after at least two prior therapies and relapsed or refractory follicular lymphoma (FL) after at least two prior systemic therapies. The indication in FL is approved under accelerated approval based on overall response rate. Continued approval for this FL indication may be contingent upon verification and description of clinical benefits in confirmatory trials.

We are also developing duvelisib for the treatment of multiple types of cancer, the most advanced of which is designed to treat patients with peripheral T-cell lymphoma (PTCL). The development of duvelisib in PTCL has been awarded Fast Track status by the FDA and a registration study is underway. During 2019, we plan to continue to further develop duvelisib through the initiation of a confirmatory study of patients with FL and other sponsored trials. We also plan to report interim data for several ongoing investigator sponsored studies (ISTs).

Our second product candidate, defactinib, is a targeted inhibitor of the Focal Adhesion Kinase (FAK) signaling pathway. FAK is a non-receptor tyrosine kinase encoded by the Protein Tyrosine Kinase-2 (PTK-2) gene that is involved in cellular adhesion and, in cancer, metastatic capability. Similar to COPIKTRA, defactinib is delivered orally and designed to be a potential therapy for patients to take at home under the advice and guidance of their physician. Defactinib is currently being investigated in combination with immunotherapeutic and other agents through ISTs. During 2019, we plan to report results from certain ongoing dose escalation combination studies involving this product.

Our operations to date have been organizing and staffing our company, business planning, raising capital, identifying and acquiring potential product candidates, undertaking preclinical studies and clinical trials for our product candidates, and initiating U.S. commercial operations following the approval of COPIKTRA. We have financed our operations to date primarily through public offerings of our common stock, sales of common stock under our at-the-market equity offering programs, our loan and security agreement executed with Hercules Capital, Inc. (Hercules) in March 2017, as amended, the upfront payments under our license and collaboration agreements with Yakult and CSPC, and the issuance of \$150.0 million aggregate principal amount of Notes in October 2018. With our U.S. commercial launch of COPIKTRA on September 24, 2018, we have recently begun financing a portion of our operations through product revenue.

As of June 30, 2019, we had an accumulated deficit of \$455.9 million. Our net loss was \$42.2 million, \$80.3 million, \$18.4 million and \$39.4 million for the three and six months ended June 30, 2019 and 2018, respectively. We expect to incur significant expenses and increasing operating losses for the foreseeable future as a result of our commercialization of COPIKTRA and the continued research and development of all of our product candidates. We will need to generate significant revenues to achieve profitability, and we may never do so.

We believe that we may have sufficient funds to meet our obligations within the next twelve months from the date of issuance of these condensed consolidated financial statements. However, COPIKTRA is our only approved product and our business currently depends heavily on its successful commercialization. Successful commercialization of an approved product is an expensive and uncertain process. These uncertainties and risk factors raise substantial doubt about our ability to continue as a going concern.

## **CRITICAL ACCOUNTING POLICIES AND SIGNIFICANT JUDGMENTS AND ESTIMATES**

We believe that several accounting policies are important to understanding our historical and future performance. We refer to these policies as “critical” because these specific areas generally require us to make judgments and estimates about matters that are uncertain at the time we make the estimate, and different estimates—which also would have been reasonable—could have been used, which would have resulted in different financial results.

The critical accounting policies we identified in our most recent Annual Report on Form 10-K for the fiscal year ended December 31, 2018 related to accrued research and development expenses, stock-based compensation, revenue recognition, collaborative arrangements, accounts receivable, inventory and intangible assets. During the six months ended June 30, 2019, there were no material changes to the significant accounting policies, except for the adoption of Accounting Standards Codification (ASC) 842, *Leases*, issued by the Financial Accounting Standards Board (the FASB), which is detailed below.

### ***Revenue Recognition***

Under ASC 606, an entity recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the entity expects to receive in exchange for those goods or services. To determine revenue recognition for arrangements that an entity determines are within the scope of ASC 606, the entity performs the following five steps: (i) identify the contract(s) with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) the entity satisfies a performance obligation. We only apply the five-step model to contracts when it is probable that we will collect the consideration we are entitled to in exchange for the goods or services we transfer to the customer. At contract inception, once the contract is determined to be within the scope of ASC 606, we assess the goods or services promised within each contract and determine which goods or services are performance obligations, and assess whether each promised good or service is distinct. We then recognize as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied.

*Product Revenue, Net* – We sell COPIKTRA to a limited number of specialty pharmacies and specialty distributors in the United States. These customers subsequently resell COPIKTRA either directly to patients, or to community hospitals or oncology clinics with in-office dispensaries who in turn distribute COPIKTRA to patients. In addition to distribution agreements with customers, we also enter into arrangements with (1) certain government

agencies and various private organizations (Third-Party Payers), which may provide for chargebacks or discounts with respect to the purchase of COPIKTRA, and (2) Medicare and Medicaid, which may provide for certain rebates with respect to the purchase of COPIKTRA.

We recognize revenue on sales of COPIKTRA when a customer obtains control of the product, which occurs at a point in time (typically upon delivery). Product revenues are recorded at the wholesale acquisition costs, net of applicable reserves for variable consideration. Components of variable consideration include trade discounts and allowances, Third-Party Payer chargebacks and discounts, government rebates, other incentives, such as voluntary co-pay assistance, product returns, and other allowances that are offered within contracts between us and customers, payors, and other indirect customers relating to our sale of COPIKTRA. These reserves, as detailed below, are based on the amounts earned, or to be claimed on the related sales, and are classified as reductions of accounts receivable or a current liability. These estimates take into consideration a range of possible outcomes based upon relevant factors such as, customer contract terms, information received from third-parties regarding the anticipated payor mix for COPIKTRA, known market events and trends, industry data, and forecasted customer buying and payment patterns. Overall, these reserves reflect our best estimates of the amount of consideration to which we are entitled with respect to sale made.

The amount of variable consideration which is included in the transaction price may be constrained and is included in the net sales price only to the extent that it is probable that a significant reversal in the amount of the cumulative revenue recognized under contracts will not occur in a future period. Our analyses contemplate the application of the constraint in accordance with ASC 606. For the three and six months ended June 30, 2019, we determined a material reversal of revenue would not occur in a future period for the estimates detailed below and, therefore, the transaction price was not reduced further. Actual amounts of consideration ultimately received may differ from our estimates. If actual results in the future vary from our estimates, we will adjust these estimates, which would affect net product revenue and earnings in the period such variances become known.

**Trade Discounts and Allowances:** We generally provide customers with invoice discounts on sales of COPIKTRA for prompt payment, which are explicitly stated in our contracts and are recorded as a reduction of revenue in the period the related product revenue is recognized. In addition, we compensate our specialty distributor customers for sales order management, data, and distribution services. We have determined such services are not distinct from our sale of COPIKTRA to the specialty distributor customers and, therefore, these payments have also been recorded as a reduction of revenue within the condensed consolidated statements of operations and comprehensive loss through June 30, 2019.

**Third-Party Payer Chargebacks, Discounts and Fees:** We execute contracts with Third-Party Payers which allow for eligible purchases of COPIKTRA at prices lower than the wholesale acquisition cost charged to customers who directly purchase the product from us. In some cases, customers charge us for the difference between what they pay for COPIKTRA and the ultimate selling price to the Third-Party Payers. These reserves are established in the same period that the related revenue is recognized, resulting in a reduction of product revenue and accounts receivable, net. Chargeback amounts are generally determined at the time of resale to the qualified Third-Party Payer by customers, and we generally issue credits for such amounts within a few weeks of the customer's notification to us of the resale. Reserves for chargebacks consist of credits that we expect to issue for units that remain in the distribution channel inventories at the end of each reporting period that we expect will be sold to Third-Party Payers, and chargebacks that customers have claimed, but for which we have not yet issued a credit. In addition, we compensate certain Third-Party Payers for administrative services, such as account management and data reporting. These administrative service fees have also been recorded as a reduction of product revenue within the condensed consolidated statements of operations and comprehensive loss through June 30, 2019.

**Government Rebates:** We are subject to discount obligations under state Medicaid programs and Medicare. These reserves are recorded in the same period the related revenue is recognized, resulting in a reduction of product revenue and the establishment of a current liability which is included in accrued expenses on the consolidated balance sheets. For Medicare, we also estimate the number of patients in the prescription drug coverage gap for whom we will owe an additional liability under the Medicare Part D program. Our liability for these rebates consists of invoices received for claims from prior quarters that have not been paid or for which an invoice has not yet been received,

estimates of claims for the current quarter, and estimated future claims that will be made for product that has been recognized as revenue, but which remains in the distribution channel inventories at the end of each reporting period.

**Other Incentives:** Other incentives which we offer include voluntary co-pay assistance programs, which are intended to provide financial assistance to qualified commercially-insured patients with prescription drug co-payments required by payors. The calculation of the accrual for co-pay assistance is based on an estimate of claims and the cost per claim that we expect to receive for product that has been recognized as revenue, but remains in the distribution channel inventories at the end of each reporting period. The adjustments are recorded in the same period the related revenue is recognized, resulting in a reduction of product revenue and the establishment of a current liability which is included as a component of accrued expenses on the condensed consolidated balance sheets.

**Product Returns:** Consistent with industry practice, we generally offer customers a limited right of return for product that has been purchased from us. We estimate the amount of our product sales that may be returned by our customers and record this estimate as a reduction of revenue in the period the related product revenue is recognized. We estimate product return liabilities using available industry data and our own sales information, including our visibility into the inventory remaining in the distribution channel.

Subject to certain limitations, our return policy allows for eligible returns of COPIKTRA for credit under the following circumstances:

- Receipt of damaged product;
- Shipment errors that were a result of an error by us;
- Expired product that is returned during the period beginning three months prior to the product's expiration and ending six months after the expiration date;
- Product subject to a recall; and
- Product that we, at our sole discretion, have specified can be returned for credit.

We have not received any returns to date.

If taxes should be collected from customers relating to product sales and remitted to governmental authorities, they will be excluded from product revenue. We expense incremental costs of obtaining a contract when incurred, if the expected amortization period of the asset that we would have recognized is one year or less. However, no such costs were incurred during the three and six months ended June 30, 2019.

*Exclusive Licenses of Intellectual Property* - We may enter into collaboration and licensing arrangements for research and development, manufacturing, and commercialization activities with collaboration partners for the development and commercialization of our product candidates, which have components within the scope of ASC 606. The arrangements generally contain multiple elements or deliverables, which may include (i) licenses, or options to obtain licenses, to our intellectual property, (ii) research and development activities performed for the collaboration partner, (iii) participation on joint steering committees, and (iv) the manufacturing of commercial, clinical or preclinical material. Payments pursuant to these arrangements typically include non-refundable, upfront payments, milestone payments upon the achievement of significant development events, research and development reimbursements, sales milestones, and royalties on product sales. The amount of variable consideration is constrained until it is probable that the revenue is not at a significant risk of reversal in a future period. The contracts into which we enter generally do not include significant financing components.

In determining the appropriate amount of revenue to be recognized as we fulfill our obligations under each of our collaboration and license agreements, we perform the following steps: (i) identification of the promised goods or services in the contract within the scope of ASC 606; (ii) determination of whether the promised goods or services are performance obligations including whether they are distinct in the context of the contract; (iii) measurement of the transaction price, including the constraint on variable consideration; (iv) allocation of the transaction price to the performance obligations; and (v) recognition of revenue when (or as) we satisfy each performance obligation. As part of the accounting for these arrangements, we must use significant judgment to determine: a) the number of performance obligations based on the determination under step (ii) above; b) the transaction price under step (iii) above; c) the stand-

alone selling price for each performance obligation identified in the contract for the allocation of transaction price in step (iv) above; and d) the measure of progress in step (v) above. We use judgment to determine whether milestones or other variable consideration, except for royalties, should be included in the transaction price as described further below.

If a license to our intellectual property is determined to be distinct from the other promises or performance obligations identified in the arrangement, we recognize revenue from non-refundable, upfront fees allocated to the license when the license is transferred to the customer and the customer is able to use and benefit from the license. In assessing whether a promise or performance obligation is distinct from the other elements, we consider factors such as the research, development, manufacturing and commercialization capabilities of the collaboration partner and the availability of its associated expertise in the general marketplace. In addition, we consider whether the collaboration partner can benefit from a promise for its intended purpose without the receipt of the remaining elements, whether the value of the promise is dependent on the unsatisfied promise, whether there are other vendors that could provide the remaining promise, and whether it is separately identifiable from the remaining promise. For licenses that are combined with other promises, we utilize judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time and, if over time, the appropriate method of measuring progress for purposes of recognizing revenue. We evaluate the measure of progress as of each reporting period and, if necessary, adjust the measure of performance and related revenue recognition. The measure of progress, and thereby periods over which revenue should be recognized, is subject to estimates by management and may change over the course of the arrangement. Such a change could have a material impact on the amount of revenue we record in future periods.

**Customer Options:** If an arrangement is determined to contain customer options that allow the customer to acquire additional goods or services such as research and development services or manufacturing services, the goods and services underlying the customer options are not considered to be performance obligations at the inception of the arrangement; rather, such goods and services are contingent on exercise of the option, and the associated option fees are not included in the transaction price. We evaluate customer options for material rights or options to acquire additional goods or services for free or at a discount. If a customer option is determined to represent a material right, the material right is recognized as a separate performance obligation at the outset of the arrangement. We allocate the transaction price to material rights based on the relative standalone selling price, which is determined based on the identified discount and the estimated probability that the customer will exercise the option. Amounts allocated to a material right are not recognized as revenue until, at the earliest, the option is exercised.

**Milestone Payments:** At the inception of each arrangement that includes milestone payments, we evaluate whether the milestones are considered probable of being achieved and estimate the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal would not occur, the associated milestone value is included in the transaction price. Milestone payments that are not within the control of us or the licensee, such as regulatory approvals, are not considered probable of being achieved until those approvals are received. We evaluate factors such as the scientific, clinical, regulatory, commercial, and other risks that must be overcome to achieve the respective milestone in making this assessment. There is considerable judgment involved in determining whether it is probable that a significant revenue reversal would not occur. At the end of each subsequent reporting period, we reevaluate the probability of achievement of all milestones subject to constraint and, if necessary, adjust our estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect revenues and earnings in the period of adjustment.

**Royalties:** For arrangements that include sales-based royalties, including milestone payments based on a level of sales, and the license is deemed to be the predominant item to which the royalties relate, we recognize revenue at the later of (i) when the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). To date, we have not recognized any royalty revenue resulting from any of our licensing arrangements.

**Collaborative Arrangements:** Contracts are considered to be collaborative arrangements when they satisfy the following criteria defined in ASC 808, *Collaborative Arrangements*: (i) the parties to the contract must actively participate in the joint operating activity and (ii) the joint operating activity must expose the parties to the possibility of significant risks and rewards, based on whether or not the activity is successful. Payments received from or made to a



partner that are the result of a collaborative relationship with a partner, instead of a customer relationship, such as co-development activities, are recorded as a reduction or increase to research and development expense, respectively.

### ***Accounts Receivable, Net***

Accounts receivable, net primarily relates to amounts due from customers, net of applicable revenue reserves, or from our license and collaboration partners. Accounts receivable are typically due within 31 days. We analyze accounts that are past due for collectability and provide an allowance for receivables when collection becomes doubtful. Given the nature and limited history of collectability of our accounts receivable, an allowance for doubtful accounts is not deemed necessary at June 30, 2019.

### ***Inventory***

We capitalize inventories manufactured in preparation for initiating sales of a product candidate when the related product candidate is considered to have a high likelihood of regulatory approval and the related costs are expected to be recoverable through sales of the inventories. In determining whether or not to capitalize such inventories, we evaluate, among other factors, information regarding the product candidate's safety and efficacy, the status of regulatory submissions and communications with regulatory authorities and the outlook for commercial sales, including the existence of current or anticipated competitive drugs and the availability of reimbursement. In addition, we evaluate risks associated with manufacturing the product candidate, including the ability of our third-party suppliers to complete the validation batches, and the remaining shelf life of the inventories. Costs associated with manufacturing product candidates prior to satisfying the inventory capitalization criteria are charged to research and development expense as incurred.

We value our inventories at the lower of cost or estimated net realizable value. We determine the cost of our inventories, which includes amounts related to materials and manufacturing overhead, on a first-in, first-out basis. We perform an assessment of the recoverability of capitalized inventory during each reporting period, and we write down any excess and obsolete inventories to their estimated realizable value in the period in which the impairment is first identified. Such impairment charges, should they occur, are recorded within cost of product revenues. The determination of whether inventory costs will be realizable requires estimates by management. If actual market conditions are less favorable than projected by management, additional write-downs of inventory may be required which would be recorded as a cost of product sales in the condensed consolidated statements of operations and comprehensive loss.

Shipping and handling costs for product shipments are recorded as incurred in cost of product revenues along with costs associated with manufacturing the product, and any inventory write-downs.

### ***Intangible Assets***

We record finite-lived intangible assets related to certain capitalized milestone payments at their fair value. These assets are amortized over their remaining useful lives, which are estimated based on the shorter of the remaining underlying patent life or the estimated useful life of the underlying product. Intangible assets are amortized using the economic consumption method if anticipated future revenues can be reasonably estimated. The straight-line method is used when future revenues cannot be reasonably estimated.

We assess our finite-lived intangible assets for impairment at least annually, or if indicators are present or changes in circumstance suggest that impairment may exist. Events that could result in an impairment, or trigger an interim impairment assessment, include the receipt of additional clinical or nonclinical data regarding one of our drug candidates or a potentially competitive drug candidate, changes in the clinical development program for a drug candidate, or new information regarding potential sales for the drug. If impairment indicators are present or changes in circumstance suggest that impairment may exist, we perform a recoverability test by comparing the sum of the estimated undiscounted cash flows of each finite-lived intangible asset to its carrying value on the condensed consolidated balance sheets. If the undiscounted cash flows used in the recoverability test are less than the carrying value, we would determine the fair value of the finite-lived intangible asset and recognize an impairment loss if the carrying value of the finite-lived intangible asset exceeds its fair value.

## Leases

Effective January 1, 2019, we adopted ASC 842. This standard requires lessees to recognize in the statement of financial position a liability to make lease payments and a right-of-use asset representing its right to use the underlying asset for the lease term for both finance and operating leases.

At the inception of an arrangement, we determine whether the arrangement is or contains a lease based on the unique facts and circumstances within the arrangement. A lease is identified where an arrangement conveys the right to control the use of identified property, plant, and equipment for a period of time in exchange for consideration. Leases which are identified within the scope of ASC 842 and which have a term greater than one year are recognized on our condensed consolidated balance sheets as right-of-use assets, lease liabilities and, if applicable, long-term lease liabilities. We have elected not to recognize leases with terms of one year or less on our condensed consolidated balance sheets. Operating lease liabilities and their corresponding right-of-use assets are recorded based on the present value of lease payments over the expected remaining lease term. However, certain adjustments to the right-of-use asset may be required for items such as initial direct costs paid or incentives received. The interest rate implicit in lease contracts is typically not readily determinable. As a result, we utilize our incremental borrowing rates to calculate the present value of lease payments. Incremental borrowing rates are the rates we incur to borrow on a collateralized basis over a similar term an amount equal to the lease payments in a similar economic environment.

In accordance with ASC 842, components of a lease are split into three categories: lease components (e.g. land, building, etc.), non-lease components (e.g. common area maintenance, maintenance, consumables, etc.), and non-components (e.g. property taxes, insurance, etc.). The fixed and in-substance fixed contract consideration (including any related to non-components) must be allocated based on fair values to the lease components and non-lease components. Although separation of lease and non-lease components is required, certain practical expedients are available. Entities may elect the practical expedient to not separate lease and non-lease components. Rather, they would account for each lease component and the related non-lease component together as a single component. We have elected to account for the lease and non-lease components of each of our operating leases as a single lease component and allocate all of the contract consideration to the lease component only. The lease component results in an operating right-of-use asset being recorded on the condensed consolidated balance sheets and amortized on a straight-line basis as lease expense.

## RESULTS OF OPERATIONS

### Comparison of the three months ended June 30, 2019 and 2018

	Three months ended June 30,			
	2019	2018	Change	% Change
Revenue:				
Product revenue, net	\$ 3,019	\$ —	\$ 3,019	100%
License and collaboration revenue	117	10,000	(9,883)	-99%
Total revenue	3,136	10,000	(6,864)	-69%
Operating expenses:				
Cost of sales - product	377	—	377	100%
Cost of sales - intangible amortization	392	—	392	100%
Research and development	11,346	12,381	(1,035)	-8%
Selling, general and administrative	29,298	15,813	13,485	85%
Amortization of acquired intangible assets	—	—	—	0%
Total operating expenses	41,413	28,194	13,219	47%
Loss from operations	(38,277)	(18,194)	(20,083)	110%
Interest income	1,268	343	925	270%
Interest expense	(5,185)	(516)	(4,669)	905%
Net loss	\$ (42,194)	\$ (18,367)	\$ (23,827)	130%

*Product revenue, net.* Product revenue, net for the three months ended June 30, 2019 (2019 Quarter) was \$3.0 million and consisted of net product sales of COPIKTRA in the United States. We began commercial sales of COPIKTRA within the United States in September 2018 following receipt of FDA marketing approval. We had no product revenue during the three months ended June 30, 2018 (2018 Quarter).

*License and collaboration revenue.* License and collaboration revenue for the 2019 Quarter was \$0.1 million compared to \$10.0 million for the 2018 Quarter. The \$9.9 million decrease was related to an upfront payment received in connection to our license and collaboration agreement with Yakult in the 2018 Quarter, partially offset by collaboration revenue of \$0.1 million related to the shipment of clinical supply of COPIKTRA to Yakult and CSPC during the 2019 Quarter.

*Costs of revenues, excluding amortization of acquired intangible assets.* Costs of revenues, excluding amortization of acquired intangible assets (cost of revenues) of approximately \$0.4 million for the 2019 Quarter, consisted of costs associated with the manufacturing of COPIKTRA, royalties owed to Infinity Pharmaceuticals, Inc. (Infinity) on such sales, and certain period costs. We expensed the manufacturing costs of COPIKTRA as operating expenses in the periods prior to July 1, 2018. In the third quarter of 2018, we began capitalizing inventory costs for COPIKTRA manufactured in preparation for our launch in the United States based on our evaluation of, among other factors, the status of the COPIKTRA New Drug Application (NDA) in the United States and the ability of our third-party suppliers to successfully manufacture commercial quantities of COPIKTRA. Certain of the costs of COPIKTRA units recognized as revenue during the 2019 Quarter were expensed prior to the September 2018 FDA marketing approval and, therefore, are not included in cost of sales during this period. We expect cost of revenues to increase in relation to product revenues as we deplete these inventories. We had no costs of revenues during the 2018 Quarter.

*Research and development expense.* Research and development expense for the 2019 Quarter was \$11.3 million compared to \$12.4 million for the 2018 Quarter. The \$1.1 million decrease was primarily related to a decrease of \$0.9 million in consulting costs and a decrease of \$0.5 million in contract research organization (CRO) costs, partially offset by an increase of \$0.3 million in personnel and occupancy costs.

We allocate the expenses related to external research and development services, such as CROs, clinical sites, manufacturing organizations and consultants by project. We use our employee and infrastructure resources across multiple research and development projects. Our project costing methodology does not allocate personnel and other indirect costs to specific clinical programs. The following table summarizes our allocation of research and development expenses to our clinical programs, including COPIKTRA and defactinib, for the 2019 Quarter and the 2018 Quarter.

	<b>Three months ended June 30,</b>	
	<b>2019</b>	<b>2018</b>
	<b>(in thousands)</b>	
COPIKTRA	\$ 6,630	\$ 7,492
Defactinib	26	875
Unallocated and other research and development expense	4,276	3,390
Unallocated stock-based compensation expense	414	624
<b>Total research and development expense</b>	<b>\$ 11,346</b>	<b>\$ 12,381</b>

The decrease in COPIKTRA related costs of \$0.9 million for the 2019 Quarter as compared to the 2018 Quarter was driven by a decrease of \$1.1 million in consulting fees as a result of activities to file an NDA in the 2018 quarter and a decrease of \$0.4 million of CRO costs as a result of site closures in our DUO Phase 3 and DYNAMO Phase 2 studies throughout 2018 and 2019 as patients continue to complete treatment, offset in part by an increase of \$0.7 million of costs related to our Phase 2 Intermittent Dosing study which commenced during the 2019 Quarter. Unallocated and other research and development expenses include \$3.0 million and \$2.1 million of personnel costs for the 2019 Quarter and the 2018 Quarter, respectively.

*Selling, general and administrative expense.* Selling, general and administrative expense for the 2019 Quarter was \$29.3 million compared to \$15.8 million for the 2018 Quarter. The increase of \$13.5 million from the 2018 Quarter to the 2019 Quarter primarily resulted from increases in personnel related costs, including non-cash stock-based

compensation, of \$6.2 million, primarily related to the hiring and staffing of our sales and commercial teams, executive and non-executive separation costs of \$2.3 million including non-cash stock-based compensation, an increase in consulting and professional fees of \$4.3 million, primarily related to commercial operations following the approval of COPIKTRA in September 2018, and an increase in travel, debt advisory, and other costs of \$0.7 million.

*Amortization of acquired intangible assets.* Amortization of acquired intangible assets for the 2019 Quarter of approximately \$0.4 million was related to the COPIKTRA finite-lived intangible asset which we recognized and began amortizing in September 2018. There was no amortization of acquired intangible assets in the 2018 Quarter.

*Interest income.* Interest income for the 2019 Quarter was \$1.3 million compared to \$0.3 million for the 2018 Quarter. The increase of \$1.0 million was primarily due to higher investment cost basis and higher interest rates on investments.

*Interest expense.* Interest expense for the 2019 Quarter was \$5.2 million compared to \$0.5 million for the 2018 Quarter. The increase of \$4.7 million was due to the issuance of the Notes in October 2018, a higher principal balance on our loan and security agreement with Hercules and the acceleration of an end of term fee related to the Hercules LSA refinancing recorded as interest expense.

#### Comparison of the six months ended June 30, 2019 and 2018

	Six months ended June 30,			% Change
	2019	2018	Change	
Revenue:				
Product revenue, net	\$ 4,690	\$ —	\$ 4,690	100%
License and collaboration revenue	117	10,000	(9,883)	-99%
Total revenue	4,807	10,000	(5,193)	-52%
Operating expenses:				
Costs of revenues, excluding amortization of acquired intangible assets	534	—	534	100%
Research and development	21,103	23,315	(2,212)	-9%
Selling, general and administrative	55,331	25,640	29,691	116%
Amortization of acquired intangible assets	785	—	785	100%
Total operating expenses	77,753	48,955	28,798	59%
Loss from operations	(72,946)	(38,955)	(33,991)	87%
Other income				
Interest income	2,765	534	2,231	418%
Interest expense	(10,115)	(996)	(9,119)	916%
Net loss	\$ (80,296)	\$ (39,417)	\$ (40,879)	104%

*Product revenue, net.* Product revenue, net for the six months ended June 30, 2019 (2019 Period) was \$4.7 million and consisted of net product sales of COPIKTRA in the United States. We began commercial sales of COPIKTRA within the United States in September 2018 following receipt of FDA marketing approval. We had no product revenue during the six months ended June 30, 2018 (2018 Period).

*License and collaboration revenue.* License and collaboration revenue for the 2019 Period was \$0.1 million compared to \$10.0 million for the 2018 Period. The \$9.9 million decrease was related to an upfront payment received in connection to our license and collaboration agreement with Yakult, partially offset by collaboration revenue of \$0.1 million related to the shipment of clinical supply of COPIKTRA to Yakult and CSPC during the 2019 Period.

*Costs of revenues, excluding amortization of acquired intangible assets.* Costs of revenues, excluding amortization of acquired intangible assets (cost of revenues) of approximately \$0.5 million for the 2019 Period, consisted of costs associated with the manufacturing of COPIKTRA, royalties owed to Infinity Pharmaceuticals, Inc. (Infinity) on such sales, and certain period costs. We expensed the manufacturing costs of COPIKTRA as operating expenses in the periods prior to July 1, 2018. In the third quarter of 2018, we began capitalizing inventory costs for COPIKTRA manufactured in preparation for our launch in the United States based on our evaluation of, among other factors, the status of the COPIKTRA New Drug Application (NDA) in the United States and the ability of our third-party suppliers to successfully manufacture commercial quantities of COPIKTRA. Certain of the costs of COPIKTRA units recognized as revenue during the 2019 Period were expensed prior to the September 2018 FDA marketing approval and, therefore, are not included in cost of sales during this period. We expect cost of revenues to increase in relation to product revenues as we deplete these inventories. We had no costs of revenues during the 2018 Period.

*Research and development expense.* Research and development expense for the 2019 Period was \$21.1 million compared to \$23.3 million for the 2018 Period. The \$2.2 million decrease was primarily related to a decrease of \$1.6 million in consulting costs and a decrease of \$1.0 million in contract research organization (CRO) costs, partially offset by an increase of \$0.2 million in personnel and occupancy costs and an increase of \$0.2 million in travel and other costs.

We allocate the expenses related to external research and development services, such as CROs, clinical sites, manufacturing organizations and consultants by project. We use our employee and infrastructure resources across multiple research and development projects. Our project costing methodology does not allocate personnel and other indirect costs to specific clinical programs. The following table summarizes our allocation of research and development expenses to our clinical programs, including COPIKTRA and defactinib, for the 2019 Period and the 2018 Period.

	Six months ended June 30,	
	2019	2018
	(in thousands)	
COPIKTRA	\$ 11,345	\$ 13,484
Defactinib	879	1,316
Unallocated and other research and development expense	8,045	7,443
Unallocated stock-based compensation expense	834	1,072
<b>Total research and development expense</b>	<b>\$ 21,103</b>	<b>\$ 23,315</b>

The decrease in COPIKTRA related costs of \$2.1 million for the 2019 Period as compared to the 2018 Period was driven by a decrease of \$2.0 million in consulting fees as a result of activities to file an NDA in the 2018 Period and a decrease of \$1.7 million of CRO costs as a result of site closures in our DUO Phase 3 and DYNAMO Phase 2 studies throughout 2018 and 2019 as patients continue to complete treatment, offset in part by an increase of \$0.8 million of costs related to our PRIMO Phase 2 study for the treatment of PTCL and an increase of \$0.7 million related to our Phase 2 Intermittent Dosing study which commenced during the 2019 Quarter. Unallocated and other research and development expenses include \$5.4 million and \$4.6 million of personnel costs for the 2019 Period and the 2018 Period, respectively.

*Selling, general and administrative expense.* Selling, general and administrative expense for the 2019 Period was \$55.3 million compared to \$25.6 million for the 2018 Period. The increase of \$29.7 million from the 2018 Period to

the 2019 Period primarily resulted from increases in personnel related costs, including non-cash stock-based compensation, of \$15.0 million, primarily related to the hiring and staffing of our sales and commercial teams, executive and non-executive separation costs of \$2.3 million including non-cash stock-based compensation, an increase in consulting and professional fees of \$10.2 million, primarily related to commercial operations following the approval of COPIKTRA in September 2018, and an increase in travel, debt advisory, and other costs of \$2.2 million.

*Amortization of acquired intangible assets.* Amortization of acquired intangible assets for the 2019 Period of approximately \$0.8 million was related to the COPIKTRA finite-lived intangible asset which we recognized and began amortizing in September 2018. There was no amortization of acquired intangible assets in the 2018 Period.

*Interest income.* Interest income for the 2019 Period was \$2.8 million compared to \$0.5 million for the 2018 Period. The increase of \$2.3 million was primarily due to higher investment cost basis and higher interest rates on investments.

*Interest expense.* Interest expense for the 2019 Period was \$10.1 million compared to \$1.0 million for the 2018 Period. The increase of \$9.1 million was due to the issuance of the Notes in October 2018, a higher principal balance and higher interest rates on our loan and security agreement with Hercules and the acceleration of an end of term fee related to the Hercules LSA refinancing recorded as interest expense.

## LIQUIDITY AND CAPITAL RESOURCES

### Sources of liquidity

We have financed our operations to date primarily through public offerings of our common stock, sales of common stock under our at-the-market equity offering programs, our loan and security agreement executed with Hercules in March 2017, as amended, the upfront payments under our license and collaboration agreements with Yakult and CSPC and the issuance of \$150.0 million aggregate principal amount of Notes in October 2018. With the commercial launch of COPIKTRA in the United States in September 2018, we have recently begun financing a portion of our operations through product revenue. In April 2019, we entered into the 2019 Term Loan Agreement with Hercules in which we received an additional \$10.0 million under the 2019 Term Loan.

As of June 30, 2019, we had \$187.3 million in cash, cash equivalents and short-term investments. We primarily invest our cash, cash equivalents and short-term investments in U.S. Government money market funds and corporate bonds and commercial paper of publicly traded companies. The Company believes that it may have sufficient funds to meet its obligations within the next twelve months from the date of issuance of these condensed consolidated financial statements.

COPIKTRA is our only approved product and our business currently depends heavily on its successful commercialization. Successful commercialization of an approved product is an expensive and uncertain process. Risks and uncertainties include those identified under Item 1A. *Risk Factors*, in our Annual Report on Form 10-K for the year ended December 31, 2018 as filed with the SEC on March 12, 2019 and in any subsequent filings with the SEC.

### Cash flows

The following table sets forth the primary sources and uses of cash for the 2019 Period and the 2018 Period (in thousands):

	<u>Six months ended June 30,</u>	
	<u>2019</u>	<u>2018</u>
Net cash (used in) provided by:		
Operating activities	\$ (73,449)	\$ (32,767)
Investing activities	46,893	3,905
Financing activities	9,769	115,619
<b>Increase (decrease) in cash, cash equivalents and restricted cash</b>	<b>\$ (16,787)</b>	<b>\$ 86,757</b>

*Operating activities.* The use of cash in both periods resulted primarily from our net losses adjusted for non-cash charges and changes in the components of working capital. The \$40.7 million increase in cash used in operating activities for the 2019 Period compared to the 2018 Period was primarily due to an increase in selling, general, and administrative expenses related to the hiring and staffing of our sales and commercial teams related to the post-launch commercial operations supporting COPIKTRA, and due to a \$10.0 million dollar license payment received from Yakult during the 2018 Period.

*Investing activities.* The cash provided by investing activities for the 2019 Period relates to the net maturities of investments of \$46.9 million. The cash provided by investing activities for the 2018 Period reflects the net maturities of investments of \$4.5 million, partially offset by net purchases of property, plant, and equipment of \$0.6 million.

*Financing activities.* The cash provided by financing activities for the 2019 Period primarily represents \$9.7 million of net proceeds as a result of the Hercules Amendment and \$0.1 million of proceeds received related to stock option exercises. The cash provided by financing activities for the 2018 Period primarily represents \$81.5 million in net proceeds from the sales of our common stock under the Underwriting Agreement and Purchase Agreement described below, \$24.3 million in net proceeds received under our at-the-market equity offering program (ATM), \$9.9 million in net proceeds received from our loan and security agreement executed with Hercules, and approximately \$0.3 million related to a sale of our common stock during December 2017.

In March 2017, we terminated the ATM established in December 2013 and established a new ATM pursuant to which we were able to offer and sell up to \$35.0 million of our common stock at then current market prices from time to time through Cantor Fitzgerald & Co. (Cantor), as sales agent. In August 2017, we amended our sales agreement with Cantor to increase the maximum aggregate offering price of shares of common stock that can be sold under the ATM to \$75.0 million.

During the three and six months ended June 30, 2019, there were no sales under the ATM. During the three and six months ended June 30, 2018, we sold 6,314,410 and 16,481,475 shares under this program for net proceeds of approximately \$23.7 million and \$24.3 million (after deducting commissions and other offering expenses). Through June 30, 2019, we have sold a total of 11,518,354 shares under this program for net proceeds of approximately \$47.3 million (after deducting commissions and other offering expenses). As of June 30, 2019, we can issue an additional \$26.6 million of gross proceeds under this program.

On March 21, 2017, we entered into a term loan facility of up to \$25.0 million with Hercules. The term loan facility is governed by a loan and security agreement, dated March 21, 2017 (the Original Loan Agreement), which was amended on January 4, 2018, March 6, 2018 and October 11, 2018 (the Amended Loan Agreement) to increase the total borrowing limit under the Original Loan Agreement from up to \$25.0 million to up to \$50.0 million (the Amended Term Loan), pursuant to certain conditions of funding.

On April 23, 2019 (the Amendment Date), we entered into the Fourth Amendment (the Amendment) to the Loan and Security Agreement with Hercules. The Amendment amends the Amended Loan Agreement (together with the Amendment, the 2019 Term Loan Agreement).

Per the terms of the Amendment, we may borrow up to an aggregate of \$75.0 million, of which \$35.0 million was outstanding immediately as of the Amendment Date (2019 Term A Loan) as a result of the existing outstanding principal of term loans of \$25.0 million under the Amended Loan Agreement being converted into the 2019 Term A Loan, and an additional \$10.0 million being drawn on the Amendment Date. The remaining \$40.0 million of borrowing capacity may be drawn in multiple tranches comprised of (i) a term loan in an amount of up to \$15.0 million upon us generating cumulative net product revenues (as defined in the 2019 Term Loan Agreement) of either (a) \$37.5 million on or before April 30, 2020 or (b) \$50.0 million on or before June 30, 2020 (2019 Term B Loan), and (ii) a term loan in an amount of up to \$25.0 million available through December 31, 2021, subject to Hercules' approval and certain other conditions specified in the 2019 Term Loan Agreement (the 2019 Term C Loan, and together with the 2019 Term A Loan and 2019 Term B Loan, the 2019 Term Loan). As of June 30, 2019, we have borrowed a total of \$35.0 million in term loans.

The 2019 Term Loan will mature on December 1, 2022 (2019 Term Loan Maturity Date). Each advance accrues interest at a floating per annum rate equal to the greater of (a) 9.75% or (b) the lesser of (i) 12.00% and (ii) the sum of (x) 9.75% plus (y) (A) the prime rate minus (B) 5.50%. The 2019 Term Loan provides for interest-only payments until April 1, 2021, which may be extended to December 1, 2021 pursuant to us generating \$40.0 million in net product revenue on a trailing six-month basis on or prior to December 31, 2020 provided that no event of default has occurred. Thereafter, amortization payments will be payable monthly in equal installments of principal and interest (subject to recalculation upon a change in prime rates).

The 2019 Term Loan is secured by a lien on substantially all of our assets, other than intellectual property and contains customary covenants and representations, including a liquidity covenant, minimum net revenue covenant, financial reporting covenant and limitations on dividends, indebtedness, collateral, investments, distributions, transfers, mergers or acquisitions, taxes, corporate changes, deposit accounts, and subsidiaries.

On the Amendment Date, we were required to pay any outstanding accrued interest as well as the final payment fee equal to 4.5% on the outstanding principal balance of the Amended Term Loan, or \$1.1 million. No prepayment charges were due as a result of executing the Amendment or conversion of the existing term loans into 2019 Term A Loans.

### **License and collaboration agreements**

#### ***Yakult***

On June 5, 2018, we entered into a license and collaboration agreement with Yakult (the Yakult Agreement), under which we granted exclusive rights to Yakult to develop and commercialize products containing duvelisib in Japan for the treatment, prevention, palliation or diagnosis of all oncology indications in humans or animals. Under the terms of the Yakult Agreement, Yakult received an exclusive right to develop and commercialize products containing duvelisib in Japan under mutually agreed upon development and commercialization plans at its own cost and expense. Yakult also received certain limited manufacturing rights in the event that we are unable to manufacture or supply sufficient quantities of duvelisib or products containing duvelisib to Yakult during the term of the Yakult Agreement. We retained all rights to duvelisib outside of Japan.

Subsequently, on February 28, 2019, we entered into a supply agreement with Yakult (the Yakult Supply Agreement), under which we agreed to provide Yakult with drug product for clinical and commercial use in accordance with the Yakult Agreement. Under the terms of the Yakult Supply Agreement, we also granted to Yakult a limited manufacturing license to fill, finish, package, and label the drug product solely for clinical and commercial purposes in Japan.

Unless earlier terminated by either party, the Yakult Agreement will expire upon the fulfillment of Yakult's royalty obligations to us for the sale of any products containing duvelisib in Japan, which royalty obligations expire, on a product-by-product basis, upon the last to occur of (a) expiration of valid claims covering such product, (b) expiration of regulatory exclusivity for such product or (c) 10 years from first commercial sale of such product. Yakult may terminate the Yakult Agreement in its entirety at any time with 180 days' written notice. Either party may terminate the Yakult Agreement in its entirety with 60 days' written notice for the other party's material breach if such party fails to cure the breach. We may terminate the Yakult Agreement if (i) Yakult fails to use commercially reasonable efforts to develop and commercialize products containing duvelisib in Japan or (ii) Yakult challenges any patent licensed by us to Yakult under the Yakult Agreement. Either party may terminate the Yakult Agreement in its entirety upon certain insolvency events involving the other party.

#### ***CSPC***

On September 25, 2018, we entered into a license and collaboration agreement with CSPC (the CSPC Agreement), under which we granted exclusive rights to CSPC to develop and commercialize products containing duvelisib in the People's Republic of China (China), Hong Kong, Macau and Taiwan (each, a Region and collectively, the CSPC Territory) for the treatment, prevention, palliation or diagnosis of all oncology indications in humans. Under



the terms of the CSPC Agreement, CSPC received an exclusive right to develop and commercialize products containing duvelisib in the CSPC Territory under mutually agreed upon development and commercialization plans at its own cost and expense. CSPC also received certain limited manufacturing rights in the event that we are unable to manufacture or supply sufficient quantities of duvelisib or products containing duvelisib to CSPC during the term of the CSPC Agreement. We retained all rights to duvelisib outside of the CSPC Territory.

Unless earlier terminated by either party, the CSPC Agreement will expire upon the fulfillment of CSPC's royalty obligations to us for the sale of any products containing duvelisib in the CSPC Territory, which royalty obligations expire, on a product-by-product basis, upon the last to occur, in each specific Region of (a) expiration of valid claims covering such product, (b) expiration of regulatory exclusivity for such product or (c) 10 years from first commercial sale of such product. CSPC may terminate the CSPC Agreement in its entirety at any time with 180 days' written notice. Either party may terminate the CSPC Agreement in its entirety with 60 days' written notice for the other party's material breach if such party fails to cure the breach. We may terminate the CSPC Agreement immediately if CSPC breaches its non-compete obligations or any of its representations and warranties or covenants under the CSPC Agreement. We may also terminate the CSPC Agreement if (i) CSPC fails to use commercially reasonable efforts to develop and commercialize products containing duvelisib in the CSPC Territory or (ii) CSPC challenges any patent licensed by us to CSPC under the CSPC Agreement. Either party may terminate the CSPC Agreement in its entirety upon certain insolvency events involving the other party.

### **Funding requirements**

We expect to continue to incur significant expenses and operating losses. We anticipate that our expenses and operating losses will continue as we:

- commercialize COPIKTRA;
- continue our ongoing clinical trials, including with COPIKTRA and defactinib;
- initiate additional clinical trials for our product candidates;
- maintain, expand and protect our intellectual property portfolio;
- acquire or in-license other products and technologies;
- hire additional clinical, development and scientific personnel;
- add operational, financial and management information systems and personnel, including personnel to support our product development and commercialization efforts; and
- establish and maintain a sales, marketing and distribution infrastructure to commercialize COPIKTRA or any products for which we may obtain marketing approval.

These factors raise substantial doubt about our ability to continue as a going concern. 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 collaborations with third parties for development and commercialization of our product candidates, we are unable to estimate the amounts of increased capital outlays and operating expenses associated with completing the development of our current product candidates. Our future capital requirements will depend on many factors, including:

- the costs and timing of commercialization activities for COPIKTRA and the product candidates for which we expect to receive marketing approval;
- the scope, progress and results of our ongoing and potential future clinical trials;
- the extent to which we acquire or in-license other product candidates and technologies;
- the costs, timing and outcome of regulatory review of our product candidates (including our efforts to seek approval and fund the preparation and filing of regulatory submissions);
- revenue received from commercial sales of COPIKTRA and our product candidates, should any of our other product candidates also receive marketing approval;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property related claims; and

- our ability to establish collaborations or partnerships on favorable terms, if at all.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and licensing arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interest of our existing stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our existing stockholders. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

## **CONTRACTUAL OBLIGATIONS AND COMMITMENTS**

The disclosure of our contractual obligations and commitments was reported in our Annual Report on Form 10-K for the year ended December 31, 2018. There have not been any material changes from the contractual obligations and commitments previously disclosed in such report, other than our entering into the Fourth Amendment to the Loan and Security Agreement with Hercules Capital, Inc. This change is more fully described in “Liquidity and Capital Resources” and Note 11, Long-Term Debt in this Quarterly Report on Form 10-Q.

## **OFF-BALANCE SHEET ARRANGEMENTS**

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined under SEC rules.

### **Item 3. Quantitative and Qualitative Disclosures About Market Risk.**

We are exposed to market risk related to changes in interest rates. We had cash, cash equivalents and short-term investments of \$187.3 million as of June 30, 2019, consisting of cash, U.S. Government money market funds, corporate bonds and commercial paper of publicly traded companies. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because most of our investments are interest bearing. Our available for sale securities are subject to interest rate risk and will fall in value if market interest rates increase. Due to the short-term duration of our investment portfolio and the low risk profile of our investments, an immediate 100 basis point change in interest rates would not have a material effect on the fair market value of our portfolio.

We contract with CROs and contract manufacturers globally which may be denominated in foreign currencies. We may be subject to fluctuations in foreign currency rates in connection with these agreements. Transactions denominated in currencies other than the functional currency are recorded based on exchange rates at the time such transactions arise. As of June 30, 2019, an immaterial amount of our total liabilities was denominated in currencies other than the functional currency.

On April 23, 2019, we entered into the 2019 Term Loan Agreement, under which we have borrowed \$35.0 million, inclusive of the original \$25.0 million borrowed under the Amended Term Loan Agreement. The 2019 Term Loan Agreement bears interest per annum equal to the greater of either (a) 9.75% or (b) the lesser of (i) 12.00% and (ii) the sum of (x) 9.75% plus (y) (A) the prime rate minus (B) 5.50%. Changes in interest rates can cause interest charges to fluctuate under the Amended Loan Agreement. A 10% increase in current interest rates would have resulted in an immaterial increase in the amount of cash interest expense for the three and six months ended June 30, 2019. As of June 30, 2019, we have borrowed \$35.0 million under the 2019 Term Loan Agreement.

The Notes bear interest at a fixed rate and therefore have minimal exposure to changes in interest rates; however, because these interest rates are fixed, we may be paying a higher interest rate, relative to market, in the future if our credit rating improves or other circumstances change.

**Item 4. Controls and Procedures.**

**Evaluation of disclosure controls and procedures**

Our management, with the participation of our Chief Executive Officer and our Chief Business and Financial Officer, evaluated the effectiveness of our disclosure controls and procedures as of June 30, 2019. The term “disclosure controls and procedures,” as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act of 1934 (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. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of June 30, 2019, our Chief Executive Officer and our Chief Business and Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

**Changes in internal control over financial reporting**

There have been no changes in our internal control over financial reporting during the three months ended June 30, 2019 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

## PART II—OTHER INFORMATION

### Item 1. Legal Proceedings.

None.

### Item 1A. Risk Factors.

You should carefully review and consider the information regarding certain factors that could materially affect our business, financial condition or future results set forth under Item 1A. (Risk Factors) in our Annual Report on Form 10-K for the fiscal year ended December 31, 2018 as filed with the SEC on March 12, 2019. There have been no material changes from the risk factors disclosed in our Annual Report on Form 10-K for the fiscal year ended December 31, 2018, other than as set forth below.

#### ***We may require additional financing to execute our operating plan and continue to operate as a going concern.***

As required under Accounting Standards Update 2014-15, *Presentation of Financial Statements-Going Concern* (ASC 205-40), we have the responsibility to evaluate whether conditions and/or events raise substantial doubt about our ability to meet our future financial obligations as they become due within one year after the date the financial statements are issued. The Company believes that it may have sufficient funds to meet its obligations within the next twelve months from the issuance of these condensed consolidated financial statements. However, this belief relies on the achievement of certain mitigation efforts, to include the successful commercialization of COPIKTRA in the United States, and potentially, the raising of additional capital and reduction of cash expenditures. The analysis under ASC 205-40, initially cannot take into consideration the potential mitigating effects of plans that have not been fully implemented as of the date the financial statements are issued. Additionally, the Company has historical losses from operations and anticipates that it will continue to incur losses as it continues the commercialization of COPIKTRA and the research and development of its product candidates. COPIKTRA is the Company's only approved product and the Company's business currently depends heavily on its successful commercialization. Successful commercialization of an approved product is an expensive and uncertain process. Accordingly, these uncertainties and risk factors meet the ASC 205-40 standard for raising substantial doubt about our ability to continue as a going concern within one year of the issuance date of our condensed consolidated financial statements. Lack of necessary funds may require us, among other things, to delay, scale back, or eliminate some or all of our planned clinical trials. Because we continue to experience net operating losses, our ability to continue as a going concern is subject to our ability to increase sales of COPIKTRA, obtain necessary capital from outside sources, including obtaining additional capital from the sale of our securities or assets, obtaining loans from financial institutions or entering into additional partnership arrangements. Our continued net operating losses increase the difficulty in obtaining such capital, and there can be no assurances that we will be able to obtain such capital on favorable terms or at all. If we are unable to raise capital when needed, we may be forced to delay, reduce or eliminate our commercial efforts for COPIKTRA or our other research and development activities for our product candidates, or ultimately not be able to continue as a going concern.

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

#### RECENT SALES OF UNREGISTERED SECURITIES

None.

#### PURCHASE OF EQUITY SECURITIES

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

**Item 3. Defaults Upon Senior Securities.**

None.

**Item 4. Mine Safety Disclosures.**

None.

**Item 5. Other Information.**

None.

**Item 6. Exhibits.**

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

**EXHIBIT INDEX**

- 31.1\* [Certification of Principal Executive Officer pursuant to Rules 13a-14\(a\) or 15d-14\(a\) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.](#)
- 31.2\* [Certification of Chief Financial Officer pursuant to Rules 13a-14\(a\) or 15d-14\(a\) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.](#)
- 32.1\* [Certification of Principal Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.](#)
- 32.2\* [Certification of Chief Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.](#)
- 99.1\* [Press Release issued by Verastem, Inc. on August 1, 2019.](#)
- 101.INS\* XBRL Instance Document
- 101.SCH\* XBRL Taxonomy Extension Schema Document
- 101.CAL\* XBRL Taxonomy Extension Calculation Linkbase Document
- 101.DEF\* XBRL Taxonomy Extension Definition Linkbase Document
- 101.LAB\* XBRL Taxonomy Extension Label Linkbase Document
- 101.PRE\* XBRL Taxonomy Extension Presentation Linkbase Document

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\* Filed or furnished herewith.



## CERTIFICATIONS

I, Brian M. Stuglik, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Verastem, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

/s/ BRIAN M. STUGLIK

Brian M. Stuglik  
Chief Executive Officer  
(Principal executive officer)

Date: August 1, 2019



## CERTIFICATIONS

I, Robert Gagnon, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Verastem, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

/s/ ROBERT GAGNON

Robert Gagnon  
*Chief Business and Financial Officer*  
*(Principal financial and accounting officer)*

Date: August 1, 2019

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

In connection with the Quarterly Report on Form 10-Q of Verastem, Inc. (the "Company") for the period ended June 30, 2019, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Brian Stuglik, Chief Executive Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to his knowledge:

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

/s/ BRIAN M. STUGLIK

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Brian M. Stuglik  
*Chief Executive Officer*  
*(Principal executive officer)*

Date: August 1, 2019

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

In connection with the Quarterly Report on Form 10-Q of Verastem, Inc. (the "Company") for the period ended June 30, 2019 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Robert Gagnon, Chief Business and Financial Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to his knowledge:

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

/s/ ROBERT GAGNON

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Robert Gagnon  
*Chief Business and Financial Officer*  
*(Principal financial and accounting officer)*

Date: August 1, 2019



## **Verastem Oncology Reports Second Quarter 2019 Financial Results and Highlights Recent Company Progress**

*Company Reports \$3.0 Million in Net Product Revenues from COPIKTRA®; Raises Product Revenue Guidance for 2019*

*Cash, Cash Equivalents and Short-Term Investments of \$187.3 Million as of June 30, 2019*

*Company to Host Conference Call Today at 4:30 PM ET*

**BOSTON, MA – Aug 1, 2019** – Verastem, Inc. (Nasdaq: VSTM), operating as Verastem Oncology (or “the Company”), focused on developing and commercializing medicines seeking to improve the survival and quality of life of cancer patients, today reported financial results for the three months ended June 30, 2019, and provided an overview of recent accomplishments and clinical development progress for duvelisib (COPIKTRA®).

“With the third full quarter of the COPIKTRA launch now complete, including the first full quarter of the follicular lymphoma (FL) marketing campaign, net sales are up 81% quarter-over-quarter.” said Dan Paterson, President and Chief Operating Officer of Verastem Oncology. “We have begun to see early signs that our physician education efforts are having an impact and overcoming the historical misperceptions that surround PI3K inhibitors, namely through strong key opinion leader engagement, increased podium presentations and numerous new requests for investigator-sponsored research. Overall, we are encouraged by the breadth of reach the commercial team is achieving with hematologic oncologists and we look forward to building on this strong momentum for the remainder of 2019.”

### **Key Second Quarter 2019 and Recent Accomplishments:**

#### ***Corporate and Financial***

- ***Brian Stuglik Appointed Chief Executive Officer and Other Leadership Changes*** – In July, Verastem Oncology announced the appointment of Brian Stuglik as Chief Executive Officer. Mr. Stuglik, who has served as a member of the Company’s Board of Directors since September 2017, succeeds Robert Forrester who stepped down in June 2019. Other leadership changes include Dan Paterson, the Company’s Chief Operating Officer, assuming the role of President and Chief Operating Officer and Rob Gagnon, the Company’s Chief Financial Officer, appointed to Chief Business and Financial Officer.
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- **Signed Exclusive License Agreement with Sanofi for the Development and Commercialization of Duvelisib in Select Eurasian Territories** – In July 2019, the Company announced its entry into an exclusive license agreement with Sanofi, under which Verastem Oncology granted exclusive rights to Sanofi to develop and commercialize products containing COPIKTRA in Russia and CIS, Turkey, the Middle East and Africa. Under the terms of the agreement, Verastem Oncology will receive an upfront payment of \$5 million (USD) and is eligible to receive aggregate payments of up to \$42 million if certain development and sales milestones are successfully achieved, plus double-digit percentage royalties based on future net sales of COPIKTRA in the licensed territories. In exchange, Sanofi received exclusive rights to develop and commercialize COPIKTRA and hold the marketing authorization and product license for COPIKTRA in the licensed territories. Additionally, Sanofi will have the right to collaborate with Verastem Oncology on certain global development and clinical trial activities.

#### **COPIKTRA (duvelisib)**

- **Ongoing Commercialization of COPIKTRA in the United States (U.S.)** – Verastem Oncology continued the ongoing launch of COPIKTRA, an oral inhibitor of phosphoinositide 3-kinase (PI3K), and the first approved dual inhibitor of PI3K-delta and PI3K-gamma, in the U.S. for the treatment of adult patients with relapsed or refractory chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL) after at least two prior therapies or relapsed or refractory FL after at least two prior systemic therapies. Accelerated approval in FL was based on overall response rate and continued approval may be contingent upon confirmatory trials, the first of which is expected to start in 2019. During the second quarter of 2019, the number of prescribing physicians increased by over 50% and the Company has now achieved reimbursement coverage for COPIKTRA with virtually all the targeted insurance plans. COPIKTRA contains a BOXED WARNING and Verastem Oncology has implemented a Risk Evaluation and Mitigation Strategy to provide appropriate dosing and safety information to better support physicians in managing their patients on COPIKTRA.
  - **Presented COPIKTRA Data at the American Society of Clinical Oncology (ASCO) 2019 Annual Meeting** – In early June, an abstract was presented at ASCO 2019 that highlighted dose modification data from the Phase 3 DUO study evaluating COPIKTRA in patients with relapsed or refractory CLL after at least two prior therapies. This is the same indication for which COPIKTRA received approval from the FDA in September 2018. These new data demonstrated that dose modifications of COPIKTRA may be used to effectively manage treatment-emergent adverse events, while allowing patients to remain on therapy. Specifically, the data suggest that dosing interruptions of a median of 15 days resulted in similar response rates and progression-free survival to the 16.4 months shown in the COPIKTRA label. The data also showed that when adverse events of special interest (AESIs) occur, they tend to appear in the first few months of treatment, followed by a proportionate decrease in the number of patients experiencing AESIs.
  - **Presented COPIKTRA Data at the European Hematology Association (EHA) 2019 Annual Meeting** – In June, two posters were presented at EHA 2019. The first poster described results from a post-hoc analysis evaluating the effect of COPIKTRA on lymphocytosis in patients with relapsed or refractory CLL/SLL from the Phase 3 DUO study. In this analysis, treatment with COPIKTRA rapidly increased lymphocytes and resulted in shrinkage of lymph nodes, with 86% of patients achieving a lymph node response. The data were similar in high-risk patients. COPIKTRA also resulted in resolution of lymphocytosis at up to 21 weeks. The other poster was an encore presentation of the COPIKTRA dose modification data from ASCO 2019.
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· **Presented Supportive Duvelisib Data in Relapsed or Refractory PTCL at the 15<sup>th</sup> International Congress on Malignant Lymphoma (ICML)** – In June, Dr. Steven Horwitz, MD, Memorial Sloan Kettering Cancer Center, and lead investigator of the Company's ongoing Phase 2 PRIMO study, gave an oral presentation highlighting supportive data from two Phase 1 clinical studies evaluating duvelisib in patients with relapsed or refractory PTCL. Across both studies, patients treated with duvelisib demonstrated preliminary, but compelling clinical activity, including a positive trend in response rates. The preliminary safety profile of duvelisib in patients with relapsed or refractory PTCL was considered reasonable and consistent with prior studies. The goal of the ongoing Phase 2 PRIMO study is to provide guidance on a duvelisib monotherapy dosing regimen in patients with relapsed or refractory PTCL and to further characterize its efficacy and tolerability in this population.

Other abstracts presented at ICML included an analysis of efficacy and safety of duvelisib compared to ofatumumab from the Phase 3 DUO study in patients with relapsed or refractory CLL/SLL after  $\geq 2$  prior therapies, characterization of duvelisib in patients with refractory marginal zone lymphoma from the Phase 2 DYNAMO study, and an overview of preclinical data showing the potential of duvelisib in mantle cell lymphoma.

## **Second Quarter 2019 Financial Results**

Net product revenue for the three months ended June 30, 2019 (2019 Quarter) was \$3.0 million, which reflects the third full quarter of recorded sales for COPIKTRA. The Company did not have any product revenue for the three months ended June 30, 2018 (2018 Quarter) as the FDA approved COPIKTRA on September 24, 2018. License and collaboration revenue for the 2019 Quarter was \$0.1 million, compared to \$10.0 million for the 2018 Quarter. The 2018 Quarter included license revenue of \$10.0 million, related to the upfront payment received in connection with the license and collaboration agreement with Yakult in June 2018.

Research and development (R&D) expense for the 2019 Quarter was \$11.3 million, compared to \$12.4 million for the 2018 Quarter. The decrease of \$1.1 million, or 8%, was primarily related to a decrease in consulting fees as a result of activities to file a New Drug Application for COPIKTRA in the 2018 Quarter and lower R&D costs associated with the development of COPIKTRA as a result of site closures in the Company's Phase 3 DUO and Phase 2 DYNAMO studies throughout 2018 and 2019 as patients continued to complete treatment. All of these lower costs were partially offset by an increase in costs related to the Company's Phase 2 PRIMO study for the treatment of patients with relapsed or refractory PTCL.

Selling, general and administrative expense for the 2019 Quarter was \$29.3 million, compared to \$15.8 million for the 2018 Quarter. The increase of \$13.5 million, or 85%, was primarily due to higher personnel and related costs, as well as promotional and consulting costs in support of the launch of COPIKTRA which includes executive and non-executive separation costs, debt advisory and other costs of \$2.7 million.

Net loss for the 2019 Quarter was \$42.2 million, or \$0.57 per share (basic and diluted), compared to \$18.4 million, or \$0.30 per share (basic and diluted), for the 2018 Quarter.

For the 2019 Quarter, non-GAAP adjusted net loss was \$35.7 million, or \$0.48 per share, compared to non-GAAP adjusted net loss of \$16.7 million, or \$0.27 per share, for the 2018 Quarter. Please refer to the GAAP to Non-GAAP Reconciliation attached to this press release.

As of June 30, 2019, Verastem Oncology had cash, cash equivalents and short-term investments of \$187.3 million.

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## **Financial Guidance for Fiscal 2019**

Verastem Oncology is raising its full-year guidance for net product revenue of COPIKTRA. The Company now expects net product revenue of COPIKTRA to be in the range of \$12-14 million, higher than the previous estimate of \$10-12 million. This guidance is based on product revenue to date, current run rates and near-term expectations.

## **Conference Call and Webcast Information**

The Verastem Oncology management team will host a conference call and webcast today, Thursday, August 1, 2019, at 4:30 PM (ET). The call can be accessed by dialing (877) 341-5660 (U.S. and Canada) or (315) 625-3226 (international), five minutes prior to the start of the call and providing the passcode 6256817.

The live, listen-only webcast of the conference call can be accessed by visiting the investors section of the Company's website at [www.verastem.com](http://www.verastem.com). A replay of the webcast will be archived on the Company's website for 90 days following the call.

## **About Chronic Lymphocytic Leukemia/Small Lymphocytic Lymphoma**

Chronic lymphocytic leukemia (CLL) and small lymphocytic lymphoma (SLL) are cancers that affect lymphocytes and are essentially the same disease, with the only difference being the location where the cancer primarily occurs. When most of the cancer cells are located in the bloodstream and the bone marrow, the disease is referred to as CLL, although the lymph nodes and spleen are often involved. When the cancer cells are located mostly in the lymph nodes, the disease is called SLL. The symptoms of CLL/SLL include a tender, swollen abdomen and feeling full even after eating only a small amount. Other symptoms can include fatigue, shortness of breath, anemia, bruising easily, night sweats, weight loss, and frequent infections. However, many patients with CLL/SLL will live for years without symptoms. In 2018, there were approximately 200,000 patients in the United States affected by CLL/SLL with nearly 20,000 new diagnoses. While there are therapies currently available, real-world data reveals that a significant number of patients either relapse following treatment, become refractory to current agents, or are unable to tolerate treatment, representing a significant medical need. The potential of additional oral agents, particularly as a monotherapy that can be used in the general community physician's armamentarium, may hold significant value in the treatment of patients with CLL/SLL.

## **About Follicular Lymphoma**

Follicular lymphoma (FL) is typically a slow-growing or indolent form of non-Hodgkin lymphoma (NHL) that arises from B-lymphocytes, making it a B-cell lymphoma. In 2018, this lymphoma subtype accounted for 20 to 30 percent of all NHL cases, with more than 140,000 people in the United States with FL and more than 13,000 newly diagnosed patients. Common symptoms of FL include enlargement of the lymph nodes in the neck, underarms, abdomen, or groin, as well as fatigue, shortness of breath, night sweats, and weight loss. Often, patients with FL have no obvious symptoms of the disease at diagnosis. Follicular lymphoma is usually not considered to be curable, but more of a chronic disease, with patients living for many years with this form of lymphoma. The potential of additional oral agents, particularly as a monotherapy that can be used in the general community physician's armamentarium, may hold significant value in the treatment of patients with FL.

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## About Peripheral T-Cell Lymphoma

Peripheral T-cell lymphoma (PTCL) is a rare, aggressive type of non-Hodgkin lymphoma (NHL) that develops in mature white blood cells called “T cells” and “natural killer (NK) cells”<sup>1</sup> which circulate with the lymphatic system.<sup>2</sup> PTCL accounts for between 10-15% of all non-Hodgkin lymphomas (NHLs) and generally affects people aged 60 years and older.<sup>1</sup> Although there are many different subtypes of peripheral T-cell lymphoma, they often present in a similar way, with widespread, enlarged, painless lymph nodes in the neck, armpit or groin.<sup>2</sup> There is currently no established standard of care for patients with relapsed or refractory disease.<sup>1</sup>

## About COPIKTRA™ (duvelisib)

COPIKTRA is an oral inhibitor of phosphoinositide 3-kinase (PI3K), and the first approved dual inhibitor of PI3K-delta and PI3K-gamma, two enzymes known to help support the growth and survival of malignant B-cells. PI3K signaling may lead to the proliferation of malignant B-cells and is thought to play a role in the formation and maintenance of the supportive tumor microenvironment.<sup>3,4,5</sup> COPIKTRA is indicated for the treatment of adult patients with relapsed or refractory chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL) after at least two prior therapies and relapsed or refractory follicular lymphoma (FL) after at least two prior systemic therapies. COPIKTRA is also being developed by Verastem Oncology for the treatment of peripheral T-cell lymphoma (PTCL), for which it has received Fast Track status, and is being investigated in combination with other agents through investigator-sponsored studies.<sup>6</sup> For more information on COPIKTRA, please visit [www.COPIKTRA.com](http://www.COPIKTRA.com). Information about duvelisib clinical trials can be found on [www.clinicaltrials.gov](http://www.clinicaltrials.gov).

## About Verastem Oncology

Verastem Oncology (Nasdaq: VSTM) is a commercial biopharmaceutical company committed to the development and commercialization of medicines to improve the lives of patients diagnosed with cancer. We are driven by the strength, tenacity and courage of those battling cancer – single-minded in our resolve to deliver new therapies that not only keep cancer at bay but improve the lives of patients diagnosed with cancer. Because for us, it’s personal.

Our first FDA approved product is now available for the treatment of patients with certain types of indolent non-Hodgkin’s lymphoma (iNHL). Our pipeline comprises product candidates that seek to treat cancer by modulating the local tumor microenvironment. For more information, please visit [www.verastem.com](http://www.verastem.com).

## COPIKTRA™ (duvelisib) – Select Important Safety Information

### **WARNING: FATAL AND SERIOUS TOXICITIES: INFECTIONS, DIARRHEA OR COLITIS, CUTANEOUS REACTIONS, and PNEUMONITIS**

See full prescribing information for complete boxed warning.

- Fatal and/or serious infections occurred in 31% of COPIKTRA-treated patients. Monitor for signs and symptoms of infection. Withhold COPIKTRA if infection is suspected.
  - Fatal and/or serious diarrhea or colitis occurred in 18% of COPIKTRA-treated patients. Monitor for the development of severe diarrhea or colitis. Withhold COPIKTRA.
  - Fatal and/or serious cutaneous reactions occurred in 5% of COPIKTRA-treated patients. Withhold COPIKTRA.
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- Fatal and/or serious pneumonitis occurred in 5% of COPIKTRA-treated patients. Monitor for pulmonary symptoms and interstitial infiltrates. Withhold COPIKTRA.

## **WARNINGS AND PRECAUTIONS**

- Hepatotoxicity: Monitor hepatic function.
- Neutropenia: Monitor blood counts.
- Embryo-Fetal toxicity: COPIKTRA can cause fetal harm. Advise patients of potential risk to a fetus and to use effective contraception.

**ADVERSE REACTIONS:** The most common adverse reactions (> 20%) are diarrhea or colitis, neutropenia, rash, fatigue, pyrexia, cough, nausea, upper respiratory infection, pneumonia, musculoskeletal pain, and anemia.

To report SUSPECTED ADVERSE REACTIONS, contact Verastem, Inc. (Verastem) at 877-7RXVSTM or 1-877-779-8786, or U.S. Food and Drug Administration (FDA) at 1-800-FDA-1088 or [www.fda.gov/medwatch](http://www.fda.gov/medwatch).

## **DRUG INTERACTIONS**

- CYP3A inducers: Avoid co-administration with strong CYP3A inducers.
- CYP3A inhibitors: Monitor for COPIKTRA toxicities when co-administered with strong or moderate CYP3A inhibitors. Reduce COPIKTRA dose to 15 mg twice daily when co-administered with strong CYP3A4 inhibitors.
- CYP3A substrates: Monitor for signs of toxicities when co-administering COPIKTRA with sensitive CYP3A substrates.

**See full Prescribing Information for complete Boxed Warning and other important safety information.**

## **Use of Non-GAAP Financial Measures**

To supplement Verastem Oncology's condensed consolidated financial statements, which are prepared and presented in accordance with generally accepted accounting principles in the United States (GAAP), the Company uses the following non-GAAP financial measures in this press release: non-GAAP adjusted net loss and non-GAAP net loss per share. These non-GAAP financial measures exclude certain amounts or expenses from the corresponding financial measures determined in accordance with GAAP. Management believes this non-GAAP information is useful for investors, taken in conjunction with the Company's GAAP financial statements, because it provides greater transparency and period-over-period comparability with respect to the Company's operating performance and can enhance investors' ability to identify operating trends in the Company's business. Management uses these measures, among other factors, to assess and analyze operational results and trends and to make financial and operational decisions. Non-GAAP information is not prepared under a comprehensive set of accounting rules and should only be used to supplement an understanding of the Company's operating results as reported under GAAP, not in isolation or as a substitute for, or superior to, financial information prepared and presented in accordance with GAAP. In addition, these non-GAAP financial measures are unlikely to be comparable with non-GAAP information provided by other companies. The determination of the amounts that are excluded from non-GAAP financial measures is a

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matter of management judgment and depends upon, among other factors, the nature of the underlying expense or income amounts. Reconciliations between these non-GAAP financial measures and the most comparable GAAP financial measures for the three and six months ended June 30, 2019 and 2018 are included in the tables accompanying this press release after the unaudited condensed consolidated financial statements.

### **Forward looking statements notice**

This press release and the commentary in the conference call to be held today each include forward-looking statements about Verastem Oncology's strategy, future plans and prospects, including statements regarding the development and activity of Verastem Oncology's lead product COPIKTRA, and Verastem Oncology's PI3K program generally, its commercialization of COPIKTRA, the potential commercial success of COPIKTRA, including financial guidance and patient population estimates, the anticipated adoption of COPIKTRA by patients and physicians, the structure of its planned and pending clinical trials and the timeline and indications for clinical development, regulatory submissions and commercialization activities. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Each forward-looking statement is subject to risks and uncertainties that could cause actual results to differ materially from those expressed or implied in such statement.

Applicable risks and uncertainties include the risks and uncertainties, among other things, regarding: the commercial success of COPIKTRA in the United States; physician and patient adoption of COPIKTRA, including those related to the safety and efficacy of COPIKTRA; the uncertainties inherent in research and development of COPIKTRA, such as negative or unexpected results of clinical trials; whether and when any applications for COPIKTRA may be filed with regulatory authorities in any other jurisdictions; whether and when regulatory authorities in any other jurisdictions may approve any such other applications that may be filed for COPIKTRA, which will depend on the assessment by such regulatory authorities of the benefit-risk profile suggested by the totality of the efficacy and safety information submitted and, if approved, whether COPIKTRA will be commercially successful in such jurisdictions; our ability to obtain, maintain and enforce patent and other intellectual property protection for COPIKTRA and our other product candidates; the scope, timing, and outcome of any legal proceedings; decisions by regulatory authorities regarding labeling and other matters that could affect the availability or commercial potential of COPIKTRA; the fact that regulatory authorities in the U.S. or other jurisdictions, if approved, could withdraw approval; whether preclinical testing of our product candidates and preliminary or interim data from clinical trials will be predictive of the results or success of ongoing or later clinical trials; that the timing, scope and rate of reimbursement for our product candidates is uncertain; that third-party payors (including government agencies) may not reimburse for COPIKTRA; that there may be competitive developments affecting our product candidates; that data may not be available when expected; that enrollment of clinical trials may take longer than expected; that COPIKTRA or our other product candidates will cause unexpected safety events, experience manufacturing or supply interruptions or failures, or result in unmanageable safety profiles as compared to their levels of efficacy; that COPIKTRA will be ineffective at treating patients with lymphoid malignancies; that we will be unable to successfully initiate or complete the clinical development and eventual commercialization of our product candidates; that the development and commercialization of our product candidates will take longer or cost more than planned; that we may not have sufficient cash to fund our contemplated operations; that we, CSPC Pharmaceutical Group, Yakult Honsha Co., Ltd., Sanofi or Infinity Pharmaceuticals, Inc. will fail to fully perform under the duvelisib license agreements; that we may be unable to make additional draws under our debt facility or obtain adequate financing in the future through product licensing, co-promotional arrangements,

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public or private equity, debt financing or otherwise; that we will not pursue or submit regulatory filings for our product candidates, including for duvelisib in patients with chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL) or indolent non-Hodgkin lymphoma (iNHL) in other jurisdictions; and that our product candidates will not receive regulatory approval, become commercially successful products, or result in new treatment options being offered to patients.

Other risks and uncertainties include those identified under the heading "Risk Factors" in the Company's Annual Report on Form 10-K for the year ended December 31, 2018 as filed with the SEC on March 12, 2019 and in any subsequent filings with the SEC. The forward-looking statements contained in this press release reflect Verastem Oncology's views as of the date hereof, and the Company does not assume and specifically disclaims any obligation to update any forward-looking statements whether as a result of new information, future events or otherwise, except as required by law.

## References

<sup>1</sup> The Leukemia & Lymphoma Society. Peripheral T-Cell Lymphoma Facts. July 2014.

<sup>2</sup> Leukemia Foundation. <http://www.leukaemia.org.au/blood-cancers/lymphomas/non-hodgkin-lymphoma-nhl/peripheral-t-cell-lymphoma>

<sup>3</sup> Winkler D.G., Faia K.L., DiNitto J.P. et al. PI3K-delta and PI3K-gamma inhibition by IPI-145 abrogates immune responses and suppresses activity in autoimmune and inflammatory disease models. *Chem Biol* 2013; 20:1-11.

<sup>4</sup> Reif K et al. Cutting Edge: Differential Roles for Phosphoinositide 3 kinases, p110-gamma and p110-delta, in lymphocyte chemotaxis and homing. *J Immunol* 2004;173:2236-2240.

<sup>5</sup> Schmid M et al. Receptor Tyrosine Kinases and TLR/IL1Rs Unexpectedly activate myeloid cell PI3K, a single convergent point promoting tumor inflammation and progression. *Cancer Cell* 2011; 19:715-727.

<sup>6</sup> [www.clinicaltrials.gov](http://www.clinicaltrials.gov), NCT03372057

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**Verastem, Inc.**  
**Condensed Consolidated Balance Sheets**  
(in thousands)

	<u>June 30,</u> <u>2019</u>	<u>December 31,</u> <u>2018</u>
Cash, cash equivalents and investments	\$ 187,253	\$ 249,653
Accounts receivable, net	1,389	306
Inventory	294	327
Prepaid expenses and other current assets	3,410	2,973
Property and equipment, net	1,149	1,369
Intangible assets, net	20,793	21,577
Right-of-use asset, net	3,225	—
Other assets	1,028	1,031
<b>Total assets</b>	<b>\$ 218,541</b>	<b>\$ 277,236</b>
<b>Current Liabilities</b>	<b>\$ 31,204</b>	<b>\$ 37,077</b>
Long-term debt	34,673	19,506
Convertible senior notes	99,163	95,231
Lease Liability, long-term	3,694	—
Other liabilities	500	1,123
Stockholders' equity	49,307	124,299
<b>Total liabilities and stockholders' equity</b>	<b>\$ 218,541</b>	<b>\$ 277,236</b>

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**Verastem, Inc.**  
**Unaudited Condensed Consolidated Statements of Operations**  
(in thousands, except per share amounts)

	<b>Three months ended</b>		<b>Six months ended</b>	
	<b>June 30,</b>		<b>June 30,</b>	
	<b>2019</b>	<b>2018</b>	<b>2019</b>	<b>2018</b>
<b>Revenue:</b>				
Product revenue, net	\$ 3,019	\$ —	\$ 4,690	\$ —
License and collaboration revenue	117	10,000	117	10,000
<b>Total revenue</b>	<b>3,136</b>	<b>10,000</b>	<b>4,807</b>	<b>10,000</b>
<b>Operating expenses:</b>				
Cost of sales - product	377	—	534	—
Cost of sales - intangible amortization	392	—	785	—
Research and development	11,346	12,381	21,103	23,315
Selling, general and administrative	29,298	15,813	55,331	25,640
<b>Total operating expenses</b>	<b>41,413</b>	<b>28,194</b>	<b>77,753</b>	<b>48,955</b>
Loss from operations	(38,277)	(18,194)	(72,946)	(38,955)
Interest income	1,268	343	2,765	534
Interest expense	(5,185)	(516)	(10,115)	(996)
<b>Net loss</b>	<b>\$ (42,194)</b>	<b>\$ (18,367)</b>	<b>\$ (80,296)</b>	<b>\$ (39,417)</b>
Net loss per share—basic and diluted	\$ (0.57)	\$ (0.30)	\$ (1.09)	\$ (0.70)
Weighted average common shares outstanding used in computing net loss per share—basic and diluted	73,877	61,256	73,865	56,074

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**Verastem, Inc.**  
**Reconciliation of GAAP to Non-GAAP Financial Information**  
(in thousands, except per share amounts)

	<u>Three months ended June 30,</u>		<u>Six months ended June 30,</u>	
	<u>2019</u>	<u>2018</u>	<u>2019</u>	<u>2018</u>
<b>Net Loss Reconciliation</b>				
Net Loss (GAAP basis)	\$ (42,194)	\$ (18,367)	\$ (80,296)	\$ (39,417)
<b>Adjust:</b>				
Amortization of acquired intangible asset	393	—	785	—
Stock-based compensation expense	3,065	1,539	5,313	2,867
Non-cash interest, net	1,207	95	2,815	178
Severance and Other	1,780	—	1,780	—
<b>Adjusted Net Loss (non-GAAP basis)</b>	<b>\$ (35,749)</b>	<b>\$ (16,733)</b>	<b>\$ (69,603)</b>	<b>\$ (36,372)</b>
<b>Reconciliation of Net Loss Per Share</b>				
Net Loss per share – diluted (GAAP Basis)	(0.57)	(0.30)	(1.09)	(0.70)
<b>Adjust per diluted share:</b>				
Amortization of acquired intangible asset	0.01	—	0.01	—
Stock-based compensation expense	0.04	0.03	0.07	0.05
Non-cash interest, net	0.02	0.00	0.04	0.00
Severance and Other	0.02	—	0.02	—
<b>Adjusted Net Loss per share – diluted (non-GAAP Basis)</b>	<b>\$ (0.48)</b>	<b>\$ (0.27)</b>	<b>\$ (0.94)</b>	<b>\$ (0.65)</b>
Weighted average common shares outstanding used in computing net loss per share—diluted	73,877	61,256	73,865	56,074

