

Verastem, Inc.  
Form 10-Q  
November 07, 2018  
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UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10 Q

(Mark One)

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

For the quarterly period ended September 30, 2018

OR

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

For the transition period from \_\_\_\_\_ to \_\_\_\_\_

Commission file number: 001 35403

Verastem, Inc.

(Exact name of registrant as specified in its charter)

Delaware	27-3269467
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification Number)
117 Kendrick Street, Suite 500	
Needham, MA	02494
(Address of principal executive offices)	(Zip Code)

(781) 292-4200

(Registrant's telephone number, including area code)

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.

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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 November 2, 2018, there were 73,740,167 shares of Common Stock, \$0.0001 par value per share, outstanding.

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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, our intent to commercialize COPIKTRA, 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,” “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, among other things, uncertainties regarding the launch timing and commercial success of COPIKTRA in the United States; uncertainties regarding 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; 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; the risk that third-payors (including government agencies) will 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 or Infinity Pharmaceuticals, Inc. will fail to fully perform under the duvelisib license agreement; 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 under the heading “Risk Factors” in this Quarterly Report on Form 10-Q and in our Annual Report on Form 10-K for the year ended December 31, 2017 as filed with the Securities and Exchange Commission (SEC) on March 13, 2018 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.

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## PART I—FINANCIAL INFORMATION

## Item 1. Condensed Consolidated Financial Statements (unaudited).

Verastem, Inc.

## CONDENSED CONSOLIDATED BALANCE SHEETS

(in thousands, except per share amounts)

	September 30, 2018 (unaudited)	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	\$ 130,727	\$ 82,176
Short-term investments	14,912	4,496
Accounts receivable, net	10,562	—
Inventory	131	—
Prepaid expenses and other current assets	2,397	1,115
Total current assets	158,729	87,787
Property and equipment, net	1,210	861
Intangible assets, net	21,969	—
Restricted cash	242	162
Other assets	1,005	981
Total assets	\$ 183,155	\$ 89,791
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	\$ 11,249	\$ 9,186
Accrued expenses	38,664	7,942
Current portion of long-term debt	3,528	—
Total current liabilities	53,441	17,128
Non-current liabilities:		
Long-term debt	21,535	14,828
Other non-current liabilities	566	151
Total liabilities	75,542	32,107
Stockholders' equity:		
Preferred stock, \$0.0001 par value; 5,000 shares authorized, no shares issued and outstanding at September 30, 2018 and December 31, 2017, respectively	—	—
Common stock, \$0.0001 par value; 100,000 shares authorized, 73,703 and 50,801 shares issued and outstanding at September 30, 2018 and December 31, 2017,	7	5

respectively

Additional paid-in capital	471,831	360,823
Accumulated other comprehensive income (loss)	2	(2)
Accumulated deficit	(364,227)	(303,142)
Total stockholders' equity	107,613	57,684
Total liabilities and stockholders' equity	\$ 183,155	\$ 89,791

See accompanying notes to the condensed consolidated financial statements.

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Verastem, Inc.

## CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(unaudited)

(in thousands, except per share amounts)

	Three months ended September 30,		Nine months ended September 30,	
	2018	2017	2018	2017
Revenue:				
License revenue	\$ 15,000	\$ —	\$ 25,000	\$ —
Product revenue, net	508	—	508	—
Total revenue	15,508	—	25,508	—
Operating expenses:				
Costs of revenues, excluding amortization of acquired intangible assets	49	—	49	—
Research and development	11,571	17,743	34,886	35,170
Selling, general and administrative	25,426	5,394	51,066	14,582
Amortization of acquired intangible assets	31	—	31	—
Total operating expenses	37,077	23,137	86,032	49,752
Loss from operations	(21,569)	(23,137)	(60,524)	(49,752)
Interest income	763	121	1,297	416
Interest expense	(862)	(110)	(1,858)	(231)
Net loss	\$ (21,668)	\$ (23,126)	\$ (61,085)	\$ (49,567)
Net loss per share—basic and diluted	\$ (0.29)	\$ (0.61)	\$ (0.99)	\$ (1.33)
Weighted-average number of common shares used in net loss per share—basic and diluted	73,644	37,630	61,995	37,207
Net loss	\$ (21,668)	\$ (23,126)	\$ (61,085)	\$ (49,567)
Unrealized (loss) gain on available-for-sale securities	(2)	7	4	(27)
Comprehensive loss	\$ (21,670)	\$ (23,119)	\$ (61,081)	\$ (49,594)

See accompanying notes to the condensed consolidated financial statements.



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Verastem, Inc.

## CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS

(unaudited)

(in thousands)

	Nine months ended September 30,	
	2018	2017
Operating activities		
Net loss	\$ (61,085)	\$ (49,567)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation	892	428
Amortization of acquired intangible assets	31	—
Stock-based compensation expense	4,908	4,070
Amortization of deferred financing costs, debt discounts and premiums and discounts on available-for-sale marketable securities	335	170
Gain on sale of fixed assets	(79)	—
Changes in operating assets and liabilities:		
Accounts receivable, net	(10,562)	—
Inventory	(131)	—
Prepaid expenses, other current assets and other assets	(1,145)	(571)
Accounts payable	2,108	3,268
Accrued expenses and other liabilities	9,401	5,219
Net cash used in operating activities	(55,327)	(36,983)
Investing activities		
Purchases of property and equipment	(1,244)	—
Sales of property and equipment	82	—
Purchases of investments	(14,912)	(6,461)
Maturities of investments	4,500	45,905
Net cash (used in) provided by investing activities	(11,574)	39,444
Financing activities		
Proceeds from long-term debt, net	9,900	2,386
Deferred debt financing costs	—	(138)
Proceeds from the exercise of stock options	637	91
Proceeds from the issuance of common stock, net	105,156	14,121
Net cash provided by financing activities	115,693	16,460
Increase in cash, cash equivalents and restricted cash	48,792	18,921
Cash, cash equivalents and restricted cash at beginning of period	82,338	32,511
Cash, cash equivalents and restricted cash at end of period	\$ 131,130	\$ 51,432
Supplemental disclosure of non-cash investing activities		
Acquired intangible assets included in intangible assets, net and accrued expenses	\$ 22,000	\$ —

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Supplemental disclosure of non-cash financing activities

Common stock issuance costs included in accounts payable and accrued expenses	\$ 15	\$ —
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See accompanying notes to the condensed consolidated financial statements.

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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 hematologic cancers including chronic lymphocytic leukemia and small lymphocytic lymphoma (CLL/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, 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 are poorly served by currently available therapies.

The Company is subject to a number of risks similar to 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 successful commercialization of COPIKTRA, or any of the Company's investigational product candidates following receipt of regulatory approval and protection of proprietary technology. 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 for the foreseeable future as it continues the commercialization of COPIKTRA and the research and development of its product candidates. As of September 30, 2018, the Company had cash, cash equivalents and investments of \$145.6 million and accumulated deficit of \$364.2 million. In October 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), for net proceeds of approximately \$145.1 million. The Company expects that its cash, cash equivalents and investments will be sufficient to fund its obligations for at least twelve months from the date of 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. Accordingly, they do not include all of the information and footnotes required by GAAP for complete financial statements. 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 nine months ended September 30, 2018 are not necessarily indicative of the results that may be expected for any other interim period or for the year ending December 31, 2018. 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, 2017 as filed with the Securities and Exchange Commission (SEC) on March 13, 2018.

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### Significant Accounting Policies

The significant accounting policies identified in the Company's Annual Report on Form 10-K for the year ended December 31, 2017 that require the Company to make estimates and assumptions include accrued research and development expenses and stock-based compensation. During the nine months ended September 30, 2018, there were no material changes to the significant accounting policies, except for the adoption of Accounting Standards Codification (ASC) 606, Revenue from Contracts with Customers, issued by the Financial Accounting Standards Board (the FASB), as well as significant accounting policies over revenue recognition, collaborative arrangements, accounts receivable, inventory and intangible assets, each of which is detailed below.

#### Revenue Recognition

Effective January 1, 2018, the Company adopted ASC 606. This standard applies to all contracts with customers, except for contracts that are within the scope of other standards, such as leases, insurance, collaboration arrangements and financial instruments. 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 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 (collectively, Customers). 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 nine months ended September 30, 2018, the Company determined a material reversal of revenue would not occur in a future period for the estimates detailed

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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 through September 30, 2018.

**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 through September 30, 2018.

**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.



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The Company's limited 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 and believes that returns of its products will be minimal.

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 nine months ended September 30, 2018.

**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 (1) licenses, or options to obtain licenses, to the Company's intellectual property, (2) research and development activities performed for the collaboration partner, (3) participation on joint steering committees, and (4) 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 future 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

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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.

For a complete discussion of the Company's accounting for its license and collaboration agreements, see Note 14, License and collaboration agreements.

#### Accounts Receivable, Net

Accounts receivable, net primarily relates to amounts due from Customers, net of applicable revenue reserves, or from the Company's license and collaboration partners. Accounts receivable are typically due within 31 days. The Company analyzes accounts that are past due for collectability and provides an allowance for receivables when collection becomes doubtful. Given the nature and limited history of collectability of the Company's accounts receivable, an allowance for doubtful accounts is not deemed necessary at September 30, 2018.

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### Inventory

The Company capitalizes 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, the Company evaluates, 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, the Company evaluates risks associated with manufacturing the product candidate, including the ability of the Company's 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.

The Company values its inventories at the lower of cost or estimated net realizable value. The Company determines the cost of its inventories, which includes amounts related to materials and manufacturing overhead, on a first-in, first-out basis. The Company performs an assessment of the recoverability of capitalized inventory during each reporting period, and it writes 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 revenues 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

The Company records 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.

The Company assesses its 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 the

Company's 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, the Company performs 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, the Company 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.

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Recently Issued Accounting Standards Updates

In August 2018, the FASB issued Accounting Standards 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 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 is 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 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 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. 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 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. The Company's analysis includes, but is not limited to, reviewing existing leases, reviewing other service agreements for embedded leases, establishing policies and procedures, assessing potential disclosures and evaluating the impact of adoption on the Company's condensed consolidated financial statements.



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Recently Adopted Accounting Standards Updates

In May 2017, the FASB issued ASU 2017-09, Compensation – Stock Compensation (Topic 718): Scope of Modification Accounting. ASU 2017-09 provides guidance about which changes to the terms or conditions of a share-based award require an entity to apply modification accounting under Topic 718. Specifically, an entity would not apply modification accounting if the fair value, vesting conditions and classification of the awards are the same immediately before and after a modification. ASU 2017-09 was effective for annual and interim periods beginning after December 15, 2017, with early adoption permitted. The Company adopted this standard prospectively effective January 1, 2018. The adoption of this ASU did not have an effect on the Company's condensed consolidated financial statements or related disclosures.

In November 2016, the FASB issued ASU 2016-18, Statement of Cash Flows (Topic 230): Restricted Cash. ASU 2016-18 requires that a statement of cash flows explain the change during the period in the total of cash, cash equivalents and amounts generally described as restricted cash or restricted cash equivalents. Amounts generally described as restricted cash and restricted cash equivalents should be included with cash and cash equivalents when reconciling the beginning-of-period and end-of-period total amounts shown on the statement of cash flows. ASU 2016-18 was effective for annual and interim periods beginning after December 15, 2017, with early adoption permitted. The Company adopted this standard effective January 1, 2018. Upon adoption of ASU 2016-18, the Company applied the retrospective transition method for each period presented and included approximately \$162,000 of restricted cash in the beginning-of-period and end-of-period cash, cash equivalents and restricted cash balance reflected in the condensed consolidated statements of cash flows for the nine months ended September 30, 2017. A reconciliation of cash, cash equivalents and restricted cash for each period presented is provided in Note 3 to the condensed consolidated financial statements.

In August 2016, the FASB issued ASU 2016-15, Statement of Cash Flows (Topic 230): Classification of Certain Cash Receipts and Cash Payments. ASU 2016-15 adds or clarifies guidance on the classification of certain cash receipts and payments in the statement of cash flows. The standard was effective for annual and interim periods beginning after December 15, 2017, with early adoption permitted. The Company adopted this standard effective January 1, 2018. The adoption of this ASU did not have an effect on the Company's condensed consolidated financial statements or related disclosures.

In May 2014, the FASB issued ASU 2014-09, Revenue from Contracts with Customers (Topic 606) which amends the guidance for accounting for revenue from contracts with customers. This ASU supersedes the revenue recognition requirements in ASC Topic 605, Revenue Recognition. In 2015 and 2016, the FASB issued additional ASUs related to ASC 606 that delayed the effective date of the guidance and clarified various aspects of the new revenue guidance, including principal versus agent considerations, identifying performance obligations, and licensing, and they include other improvements and practical expedients. The Company adopted this new standard on January 1, 2018 using the full retrospective method. There was no change to the Company's condensed consolidated financial statements as a result of the adoption.

## 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):

	September 30, 2018	December 31, 2017
Cash and cash equivalents	\$ 130,727	\$ 82,176
Restricted cash (included in prepaid expenses and other current assets)	161	—
Restricted cash	242	162
Total cash, cash equivalents and restricted cash	\$ 131,130	\$ 82,338

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Amounts included in restricted cash represent cash held to collateralize outstanding letters of credit in the amount of approximately \$403,000 and \$162,000 as of September 30, 2018 and December 31, 2017, respectively, provided as a security deposit for the Company's office space located in Needham, Massachusetts.

## 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	September 30, 2018			
	Total	Level 1	Level 2	Level 3
Financial assets				
Cash equivalents	\$ 129,309	\$ 100,006	\$ 29,303	\$ —
Short-term investments	14,912	—	14,912	—
Total financial assets	\$ 144,221	\$ 100,006	\$ 44,215	\$ —

Description	December 31, 2017			
	Total	Level 1	Level 2	Level 3
Financial assets				
Cash equivalents	\$ 80,894	\$ 75,478	\$ 5,416	\$ —
Short-term investments	4,496	—	4,496	—

Total financial assets	\$ 85,390	\$ 75,478	\$ 9,912	\$ —
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The Company's cash equivalents and investments are comprised of U.S. Government money market funds, government-sponsored enterprise securities, and corporate bonds and commercial paper of publicly traded companies. These 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 September 30, 2018 and December 31, 2017.

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## Fair Value of Financial Instruments

The fair value of the Company's long-term debt is determined using a discounted cash flow analysis using 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 September 30, 2018 and December 31, 2017 was approximately \$25.1 million and \$14.8 million, respectively. At September 30, 2018, the Company estimates that the fair value of its long-term debt, including the current portion, was approximately \$26.9 million. The fair value of the Company's long-term debt was determined using Level 3 inputs.

## 5. Investments

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

	September 30, 2018			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Cash and cash equivalents:				
Cash and money market accounts	\$ 101,424	\$ —	\$ —	\$ 101,424
Government-sponsored enterprise securities (due within 90 days)	9,987	—	—	9,987
Corporate bonds and commercial paper (due within 90 days)	19,318	—	(2)	19,316
Total cash and cash equivalents	\$ 130,729	\$ —	\$ (2)	\$ 130,727
Investments:				
Corporate bonds and commercial paper (due within 1 year)	\$ 14,908	\$ 4	\$ —	\$ 14,912
Total investments	\$ 14,908	\$ 4	\$ —	\$ 14,912
Total cash, cash equivalents and investments	\$ 145,637	\$ 4	\$ (2)	\$ 145,639

	December 31, 2017			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Cash and cash equivalents:				
Cash and money market accounts	\$ 76,760	\$ —	\$ —	\$ 76,760
Corporate bonds and commercial paper (due within 90 days)	5,418	\$ —	\$ (2)	\$ 5,416

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Total cash and cash equivalents	\$ 82,178	\$ —	\$ (2)	\$ 82,176
Investments:				
Corporate bonds and commercial paper (due within 1 year)	\$ 4,496	\$ —	\$ —	\$ 4,496
Total investments	\$ 4,496	\$ —	\$ —	\$ 4,496
Total cash, cash equivalents and investments	\$ 86,674	\$ —	\$ (2)	\$ 86,672

There were no realized gains or losses on investments for the three and nine months ended September 30, 2018 or 2017, respectively. There were seven and five investments in an unrealized loss position as of September 30, 2018 and December 31, 2017, respectively. None of these investments had been in an unrealized loss position for more than 12 months as of September 30, 2018 and December 31, 2017, respectively. The aggregate unrealized loss on these securities as of September 30, 2018 and December 31, 2017 was approximately \$2,000 and \$2,000, respectively, and the fair value was \$18.3 million and \$9.9 million, respectively. The Company considered the decline in the market value for these investments 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 investments 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 September 30, 2018 and December 31, 2017, respectively.

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## 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):

	September 30, 2018	December 31, 2017
Raw materials	\$ —	\$ —
Work in process	108	—
Finished goods	23	—
Total inventories	\$ 131	\$ —

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):

	September 30, 2018	Estimated useful life
Acquired and in-licensed rights	\$ 22,000	14 years
Less: accumulated amortization	(31)	
Total intangible assets, net	\$ 21,969	

Acquired and in-licensed rights as of September 30, 2018, 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 a milestone payment of \$22.0 million to Infinity in November 2018.

The Company recorded approximately \$31,000 in amortization expense related to finite-lived intangible assets during the three and nine months ended September 30, 2018 using the straight-line methodology. Estimated future amortization expense for finite-lived intangible assets as of September 30, 2018 is approximately \$392,000 for the remainder of 2018 and approximately \$1.6 million per year thereafter.

## 8. Accrued expenses

Accrued expenses consist of the following (in thousands):

	September 30, 2018	December 31, 2017
Infinity milestone	\$ 22,000	\$ —
Contract research organization costs	7,301	3,774
Compensation and related benefits	5,916	2,622
Commercialization costs	1,673	131
Professional fees	720	617
Consulting fees	519	448
Other	535	350
Total accrued expenses	\$ 38,664	\$ 7,942



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## 9. Long-term debt

On March 21, 2017 (Closing Date), Verastem, Inc. (the Borrower) 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 and March 6, 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 Term Loan), pursuant to certain conditions of funding.

As of September 30, 2018, the Company has borrowed a total of \$25.0 million in term loans. The availability of the remaining \$25.0 million of borrowing capacity under the Amended Loan Agreement is subject to Hercules' sole discretion and may be drawn as term loans (each a Term F Loan Advance) in minimum increments of \$5.0 million.

The Term Loan will mature on December 1, 2020 (Loan Maturity Date). Each advance accrues interest at a floating per annum rate equal to the greater of either (a) 10.5% or (b) the lesser of (i) 12.75% and (ii) the sum of (x) 10.5% plus (y) (A) the prime rate minus (B) 4.5%. The Term Loan provided for interest-only payments until November 1, 2018, which was extended to May 1, 2019 pursuant to the Amended Loan Agreement upon the Borrower's receipt of a minimum of \$20.0 million in cash proceeds from a sale of equity securities in December 2017. Thereafter, amortization payments will be payable monthly in 20 installments of principal and interest (subject to recalculation upon a change in prime rates).

The Term Loan is secured by a lien on substantially all of the assets of the Borrower, other than intellectual property, and contains customary covenants and representations.

The Company assessed all terms and features of the Amended 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 Amended Loan Agreement, including put and call features. The Company determined that all features of the Amended 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 September 30, 2018.

The future principal payments under the Amended Loan Agreement are as follows as of September 30, 2018 (in thousands):

Remainder of 2018	\$ —
2019	5,984
2020	19,016
Total principal payments	\$ 25,000



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## 10. Product revenue reserves and allowances

As of September 30, 2018, 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 nine months ended September 30, 2018 (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, 2017	\$ —	\$ —	\$ —	\$ —	\$ —
Provision related to sales in the current year	27	72	29	1	129
Adjustments related to prior period sales	—	—	—	—	—
Credits and payments made	—	—	—	—	—
Ending balance at September 30, 2018	\$ 27	\$ 72	\$ 29	\$ 1	\$ 129

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.

## 11. Net loss per share

Basic and diluted 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, without consideration for common stock equivalents. The Company's potentially dilutive shares, which include outstanding stock options and restricted stock units (RSUs), are considered to be common stock equivalents and are only included in the calculation of diluted net loss per share when their effect is dilutive.

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 September 30,		Nine months ended September 30,	
	2018	2017	2018	2017
Outstanding stock options	12,915,463	8,431,355	12,915,463	8,431,355
Outstanding restricted stock units	316,875	—	316,875	—
Total potentially dilutive securities	13,232,338	8,431,355	13,232,338	8,431,355



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## 12. Stock based compensation

## Stock options

A summary of the Company's stock option activity and related information for the nine months ended September 30, 2018 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, 2017	8,719,978	\$ 5.19	7.9	\$ 6,150
Granted	5,250,121	\$ 5.29		
Exercised	(331,851)	\$ 2.09		
Forfeited/cancelled	(722,785)	\$ 5.43		
Outstanding at September 30, 2018	12,915,463	\$ 5.30	8.1	\$ 37,095
Vested at September 30, 2018	5,782,349	\$ 6.13	6.7	\$ 16,154
Vested and expected to vest at September 30, 2018(1)	12,472,463	\$ 5.32	8.0	\$ 35,661

(1) This represents the number of vested options as of September 30, 2018, plus the number of unvested options expected to vest as of September 30, 2018.

The fair value of each stock option granted during the nine months ended September 30, 2018 and 2017 was estimated on the grant date using the Black-Scholes option-pricing model using the following weighted-average assumptions:

	Nine months ended September 30,			
	2018		2017	
Risk-free interest rate	2.63	%	1.98	%
Volatility	81	%	79	%
Dividend yield	—		—	
Expected term (years)	6.0		5.9	

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 two of the performance conditions had been achieved as of September 30, 2018. As a result, the Company has recognized approximately \$161,000 and \$669,000 of stock-based compensation expense during the three and nine months ended September 30, 2018, respectively, related to awards that vest upon the achievement of performance conditions.

At September 30, 2018, there was \$21.0 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 years.

#### Restricted stock units

The Company awards RSUs to employees under its 2012 Incentive Plan. 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.

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A summary of RSU activity during the nine months ended September 30, 2018 is as follows:

	Shares	Weighted-average grant date fair value per share
Outstanding at December 31, 2017	—	\$ —
Granted	336,000	\$ 5.51
Vested	—	\$ —
Forfeited	(19,125)	\$ 3.00
Outstanding at September 30, 2018	316,875	\$ 5.66

At September 30, 2018, there was approximately \$1.6 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 years.

### 13. Common stock

#### At-the-market equity offering programs

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

During the three months ended September 30, 2018, there were no sales under the at-the-market equity program. During the nine months ended September 30, 2018, the Company sold 6,481,475 shares under this program for net proceeds of approximately \$24.3 million (after deducting commissions and other offering expenses). Through September 30, 2018, the Company has 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).

#### Equity offerings

On May 16, 2018, the Company entered into an underwriting agreement with Cantor relating to the underwritten offering of 7,777,778 shares (the Shares) of the Company's common stock (Underwriting Agreement). Cantor agreed to purchase the Shares pursuant to the Underwriting Agreement at a price of \$4.31 per share. In addition, the

Company granted Cantor an option to purchase, at the public offering price less any underwriting discounts and commissions, an additional 1,166,666 shares of the Company's common stock, exercisable for 30 days from the date of the prospectus supplement. The option was exercised by Cantor in full on May 23, 2018. The aggregate proceeds from Cantor, net of underwriting discounts and offering costs, were approximately \$38.3 million.

On June 14, 2018, the Company entered into a purchase agreement with Consonance Capital Master Account L.P. and P Consonance Opportunities Ltd. (collectively, Consonance) relating to the registered offering of 7,166,666 shares of its common stock at a price of \$6.00 per share. The aggregate proceeds from Consonance, net of offering costs, were approximately \$42.8 million.



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14. License and collaboration agreements

Yakult Honsha Co., Ltd. (Yakult)

On June 5, 2018, the Company entered into a license and collaboration agreement (the Agreement) with Yakult, 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 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 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 (Global Clinical Trials) on a pro-rata basis.

Unless earlier terminated by either party, the 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 Agreement in its entirety at any time with 180 days' written notice. Either party may terminate the 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 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 Agreement. Either party may terminate the Agreement in its entirety upon certain insolvency events involving the other party.

The Company first assessed the Agreement under ASC 808 to determine whether the Agreement (or part of the Agreement) represents a collaborative arrangement based on the risks and rewards and activities of the parties pursuant to the Agreement. The Company accounts for collaborative arrangements (or elements within the contract that are deemed part of a collaborative arrangement), which represent a collaborative relationship and not a customer relationship, outside the scope of ASC 606. For a component of the Agreement, the Company concluded that both the Company and Yakult are exposed to significant risks while developing duvelisib and ultimately would share in the reward upon successful commercialization of duvelisib. The Company then considered each remaining component in the Agreement to determine if ASC 606 should be applied to those components. Generally, the components in the Agreement fall under one of two potential research and development activities: (i) the parties' joint participation in Global Clinical Trials and (ii) the territory-specific development of duvelisib.

For the parties' participation in the Global Clinical Trials, the Company concluded that the research and development activities and payments related to such activities are not within the scope of ASC 606 as Yakult is not a customer of the Company with regards to these activities in the context of the Agreement. As such, costs incurred to execute the Global Clinical Trials will be recorded as research and development expense and payments received from Yakult related to such will be recorded as a reduction of research and development expense.

For Territory-specific activities, the Company concluded that Yakult is a customer with regard to this component in the context of the Agreement. As such, the Territory-specific component and all related payments are within the scope of ASC 606.

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The Company determined that there were two material promises associated with the territory-specific activities: (i) an exclusive license to develop and commercialize duvelisib in the territory and (ii) the initial technology transfer. The Company determined that the exclusive license and initial technology transfer were not distinct from another, as the license has limited value without the initial technology. Therefore, the exclusive license and initial technology transfer are combined as a single performance obligation. The Company evaluated the option rights for manufacturing and supply services to determine whether they represent material rights to Yakult and concluded that the options were not issued at a significant and incremental discount and therefore do not represent material rights. As such, they are not performance obligations at the outset of the arrangement. Based on this assessment, the Company concluded one performance obligation exists at the outset of the Agreement: the exclusive license combined with the initial technology transfer.

The Company determined that the upfront payment of \$10.0 million constitutes the transaction price as of the outset of the Agreement. Future potential milestone payments were fully constrained as the risk of significant revenue reversal related to these amounts has not yet been resolved. The achievement of the future potential milestones is not within the Company's control and is subject to certain research and development success or regulatory approvals and therefore carry significant uncertainty. The Company will reevaluate the likelihood of achieving future milestones at the end of each reporting period. As all performance obligations have been satisfied, if the risk of significant revenue reversal is resolved, any future milestone revenue from the arrangement will be added to the transaction price (and thereby recognized as revenue) in the period the risk is relieved.

The Company satisfied the performance obligation upon delivery of the license and initial technology transfer and recognized the upfront payment of \$10.0 million as license revenue during the three months ended June 30, 2018.

### CSPC Pharmaceutical Group Limited (CSPC)

On July 26, 2018, the Company and CSPC entered into an Exclusivity Agreement which granted CSPC the exclusive right to negotiate a licensing agreement with the Company for duvelisib in China. CSPC paid the Company a non-refundable exclusivity fee of \$5.0 million in August 2018 (Exclusivity Fee) which was creditable against any payments agreed to under the terms of a potential definitive license agreement.

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 (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.

As of September 30, 2018, CSPC became obligated to pay the Company an aggregate upfront, non-refundable payment of \$15.0 million, less the previously paid \$5.0 million Exclusivity Fee, resulting in an outstanding payment due of \$10.0 million, which is included in accounts receivable, net within the condensed consolidated balance sheets.

The remaining \$10.0 million upfront payment was paid in full in November 2018. The Company is also 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 (Global Clinical Trials) on a pro-rata basis.

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

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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. 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 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 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.

The Company first assessed the CSPC Agreement under ASC 808 to determine whether the CSPC Agreement (or part of the CSPC Agreement) represents a collaborative arrangement based on the risks and rewards and activities of the parties pursuant to the CSPC Agreement. The Company accounts for collaborative arrangements (or elements within the contract that are deemed part of a collaborative arrangement), which represent a collaborative relationship and not a customer relationship, outside the scope of ASC 606. For a component of the CSPC Agreement, the Company concluded that both the Company and CSPC are exposed to significant risks while developing duvelisib and ultimately would share in the reward upon successful commercialization of duvelisib. The Company then considered each remaining component in the CSPC Agreement to determine if ASC 606 should be applied to those components. Generally, the components in the CSPC Agreement fall under one of two potential research and development activities: (i) the parties' joint participation in Global Clinical Trials and (ii) the territory-specific development of duvelisib.

For the parties' participation in the Global Clinical Trials, the Company concluded that the research and development activities and payments related to such activities are not within the scope of ASC 606 as CSPC is not a customer of the Company with regards to these activities in the context of the CSPC Agreement. As such, costs incurred to execute the Global Clinical Trials will be recorded as research and development expense and payments received from CSPC related to such will be recorded as a reduction of research and development expense.

For CSPC Territory-specific activities, the Company concluded that CSPC is a customer with regard to this component in the context of the CSPC Agreement. As such, the CSPC Territory-specific component and all related payments are within the scope of ASC 606.

The Company determined that there were two material promises associated with the territory-specific activities: (i) an exclusive license to develop and commercialize duvelisib in the territory and (ii) the initial technology transfer. The Company determined that the exclusive license and initial technology transfer were not distinct from another, as the license has limited value without the initial technology. Therefore, the exclusive license and initial technology transfer are combined as a single performance obligation. The Company evaluated the option rights for manufacturing and supply services to determine whether they represent material rights to CSPC and concluded that the options were not issued at a significant and incremental discount and therefore do not represent material rights. As such, they are not performance obligations at the outset of the arrangement. Based on this assessment, the Company concluded one performance obligation exists at the outset of the CSPC Agreement: the exclusive license combined with the initial technology transfer.

The Company determined that the upfront payment of \$15.0 million constitutes the transaction price as of the outset of the CSPC Agreement. Future potential milestone payments were fully constrained as the risk of significant revenue reversal related to these amounts has not yet been resolved. The achievement of the future potential milestones is not within the Company's control and is subject to certain research and development success or regulatory approvals and therefore carry significant uncertainty. The Company will reevaluate the likelihood of achieving future milestones at the end of each reporting period. As all performance obligations have been satisfied, if the risk of significant revenue reversal is resolved, any future milestone revenue from the arrangement will be added to the transaction price (and

thereby recognized as revenue) in the period the risk is relieved.

The Company satisfied the performance obligation upon delivery of the license and initial technology transfer and recognized the upfront payment of \$15.0 million as license revenue during the three months ended September 30, 2018.

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## 15. Income taxes

The Company did not record a federal or state income tax provision or benefit for the three and nine months ended September 30, 2018 and 2017 due to the expected loss before income taxes to be incurred for the years ended December 31, 2018 and 2017, as well as the Company's continued maintenance of a full valuation allowance against its net deferred tax assets.

## 16. Commitments and contingencies

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 \$660,000, which increases during the lease term to \$1.1 million for the last twelve-month period. The deferred rent obligation is included in accrued expenses (current portion) and other liabilities (noncurrent portion) in the condensed consolidated balance sheets. The Company has also agreed to pay its proportionate share of increases in operating expenses and property taxes for the building in which the leased space is located.

The minimum aggregate future lease commitments as of September 30, 2018 are as follows (in thousands):

Remainder of 2018	\$ 165
2019	716
2020	971
2021	1,020
2022	1,041
Thereafter	2,600
Total	\$ 6,513

In conjunction with the execution of the Amended Lease Agreement, the Company increased its security deposit by increasing its existing letter of credit to approximately \$403,000. The amount is included in prepaid expenses and other current assets and restricted cash on the condensed consolidated balance sheets as of September 30, 2018.

## 17. 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:

## Hercules Amendment

On October 11, 2018, the Company entered into Amendment No. 3 to the Amended Loan Agreement (the Third Amendment). The Third Amendment permits the Company to issue convertible notes in an aggregate principal amount of not more than \$175.0 million, provided that such convertible notes meet certain stipulations.

#### 5.00% Convertible Senior Notes Due 2048

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), for net proceeds of approximately \$145.1 million. 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



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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 (the Common Stock), 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. To the extent the Company has insufficient authorized but unissued shares to settle conversions in shares of Common Stock, the Company would be required to settle the deficiency in cash.

The Company will have the right, exercisable at its option, to cause all Notes then outstanding to be converted automatically if the "Daily VWAP" (as defined in the Indenture) per share of the Common Stock equals or exceeds 130% of the conversion price on each of at least 20 VWAP Trading Days (as defined in the Indenture), whether or not consecutive, during any 30 consecutive VWAP Trading Day period commencing on or after the date the Company first issued the 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 Notes are the Company's senior, unsecured obligations and are senior in right of payment to the Company's future indebtedness that is expressly subordinated in right of payment to the Notes; equal in right of payment with the Company's existing and future indebtedness that is not so subordinated, and effectively subordinated to the Company's existing and future secured indebtedness, to the extent of the value of the collateral securing such indebtedness. The Notes are structurally subordinated to all existing and future indebtedness and other liabilities, including trade payables, and (to the extent the Company is not a holder thereof) preferred equity, if any, of the Company's subsidiaries.

The Indenture includes customary covenants and set forth certain events of default after which the Notes may be declared immediately due and payable and set 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.

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### 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, 2017. 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, 2017.

#### 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 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, ovarian cancer, mesothelioma, 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 are poorly served by 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 and T-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 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. The indication in FL is approved under accelerated approval based on overall response rate and continued approval for this indication may be contingent upon verification and description of clinical benefits in confirmatory trials. Subsequently, on November 2, 2018, the U.S. Food and Drug Administration (FDA) confirmed that as the first sponsor to obtain marketing approval for COPIKTRA (duvelisib) for the above-referenced indications, we are entitled to seven years of orphan-drug exclusive approval pursuant to section 527 of the Federal Food, Drug and Cosmetic Act (21 U.S.C. 360cc).

COPIKTRA is also being developed by us for the treatment of peripheral T-cell lymphoma (PTCL), which has Fast Track status with the FDA, and is being investigated in combination with other agents through investigator-sponsored studies (ISTs). During 2019, we plan to continue to advance our development of COPIKTRA through the initiation of a confirmatory study of patients with FL and other sponsored trials, and the expansion of our study in patients with PTCL. Furthermore, we plan to report interim data for several ongoing ISTs and to enter into additional partnerships or collaborations for the potential commercialization of COPIKTRA outside of the United States.

We have entered into license and collaboration agreements with Yakult Honsha Co., Ltd. (Yakult) and CSPC Pharmaceutical Group Limited (CSPC), under which we granted Yakult and CSPC exclusive rights to develop and commercialize products containing duvelisib in specified territories including Japan and China, respectively, for the treatment, prevention, palliation or diagnosis of cell oncology indications in humans and animals, and we intend to enter into additional partnerships or collaborations for the potential commercialization of duvelisib outside of the

United States.

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 also delivered orally and designed to be a potential therapy for patients to take at home under the advice of their physician. Defactinib is currently being investigated in

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combination with immunotherapeutic and other agents through ISTs. During 2019, we plan to report the results from several ongoing dose escalation combination studies.

Our operations to date have consisted of organizing and staffing our company, business planning, raising capital, identifying and acquiring potential product candidates and undertaking preclinical studies and clinical trials for our product candidates. 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 5.00% Convertible Senior Notes due 2048 in October 2018. Following 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 September 30, 2018, we had an accumulated deficit of \$364.2 million. Our net loss was \$21.7 million, \$61.1 million, \$23.1 million and \$49.6 million for the three and nine months ended September 30, 2018 and 2017, respectively. We expect to incur significant expenses 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.

## 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, 2017 related to accrued research and development expenses and stock-based compensation. During the nine months ended September 30, 2018, there were no material changes to the significant accounting policies, except for the adoption of Accounting Standards Codification (ASC) 606, Revenue from Contracts with Customers, issued by the Financial Accounting Standards Board (the FASB), as well as significant accounting policies over revenue recognition, collaborative arrangements, accounts receivable, inventory and intangible assets, each of which is detailed below.

### Revenue Recognition

Effective January 1, 2018, we adopted ASC 606. This standard applies to all contracts with customers, except for contracts that are within the scope of other standards, such as leases, insurance, collaboration arrangements and financial instruments. 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 it transfers 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 those that 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 (collectively, Customers). 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

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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 nine months ended September 30, 2018, 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 September 30, 2018.

**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 services have also been recorded as a reduction of product revenue within the condensed consolidated statements of operations and comprehensive loss through September 30, 2018.

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 condensed 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,

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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.

Our limited 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 and believes that returns of our product will be minimal.

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 nine months ended September 30, 2018.

**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 (1) licenses, or options to obtain licenses, to our intellectual property, (2) research and development activities performed for the collaboration partner, (3) participation on joint steering committees, and (4) 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 future 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-

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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 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 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 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 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 risk and rewards, based on whether or not the activity is successful. Payments received from or made to a

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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 September 30, 2018.

### 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.

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## RESULTS OF OPERATIONS

Comparison of the three months ended September 30, 2018 and 2017

	Three months ended September 30,			
	2018	2017	Change	% Change
Revenue:				
License revenue	\$ 15,000	\$ —	\$ 15,000	100%
Product revenue, net	508	—	508	100%
Total revenue	15,508	—	15,508	100%
Operating expenses:				
Costs of revenues, excluding amortization of acquired intangible assets	49	—	49	100%
Research and development	11,571	17,743	(6,172)	-35%
Selling, general and administrative	25,426	5,394	20,032	371%
Amortization of acquired intangible assets	31	—	31	100%
Total operating expenses	37,077	23,137	13,940	60%
Loss from operations	(21,569)	(23,137)	1,568	-7%
Interest income	763	121	642	531%
Interest expense	(862)	(110)	(752)	684%
Net loss	\$ (21,668)	\$ (23,126)	\$ 1,458	-6%

License revenue. Revenue for the three months ended September 30, 2018 (2018 Quarter) was \$15.0 million and was related to an upfront payment pursuant to the license and collaboration agreement executed between ourselves and CSPC in September 2018. We had no license revenue during the three months ended September 30, 2017 (2017 Quarter).

Product revenue, net. We began commercial sales of COPIKTRA within the United States in September 2018, following receipt of FDA marketing approval on September 24, 2018. For the 2018 Quarter we recorded approximately \$508,000 of net product revenue. We had no product revenue during the 2017 Quarter.

Costs of revenues, excluding amortization of acquired intangible assets. Costs of revenues, excluding amortization of acquired intangible assets (cost of revenues) of approximately \$49,000 for the 2018 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 2018 Quarter, 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 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 2018 Quarter were expensed prior to the September 2018 FDA marketing approval and, therefore, are not included in cost of sales during the 2018 Quarter. We expect cost of revenues to increase in relation to product revenues as we deplete these inventories. We had no cost of revenues during the 2017 Quarter.

Research and development expense. Research and development expense for the 2018 Quarter was \$11.6 million compared to \$17.7 million for the 2017 Quarter. The \$6.1 million decrease from the 2017 Quarter to the 2018 Quarter was primarily related to a decrease of \$6.0 million in license fees related to a one-time milestone payment pursuant to the Infinity license agreement that was recognized in the 2017 Quarter and a decrease of \$1.2 million in consulting fees and other costs. These decreases were offset by an increase of \$1.1 million in personnel related costs, including non-cash stock-based compensation.

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We allocate the expenses related to external research and development services, such as contract research organizations (CROs), clinical sites, manufacturing organizations and consultants by project. The table below summarizes our allocation of research and development expenses to our clinical programs, including COPIKTRA and defactinib, for the 2018 Quarter and the 2017 Quarter. 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. These unallocated research and development expenses are summarized in the table below and include approximate personnel related costs of \$2.3 million and \$1.2 million for the 2018 Quarter and the 2017 Quarter, respectively.

	Three months ended September 30,	
	2018	2017
	(in thousands)	
COPIKTRA	\$ 6,703	\$ 13,600
Defactinib	375	807
Unallocated and other research and development expense	3,874	2,676
Unallocated stock-based compensation expense	619	660
Total research and development expense	\$ 11,571	\$ 17,743

**Selling, general and administrative expense.** Selling, general and administrative expense for the 2018 Quarter was \$25.4 million compared to \$5.4 million for the 2017 Quarter. The increase of \$20.0 million from the 2017 Quarter to the 2018 Quarter primarily resulted from increases in personnel related costs, including non-cash stock-based compensation, of \$9.7 million, primarily related to the hiring and staffing of our sales and commercial teams, consulting and professional fees of \$9.1 million, primarily related to the support of commercial launch preparation activities, and travel and other costs of \$1.2 million.

**Amortization of acquired intangible assets.** Amortization of acquired intangible assets for the 2018 Quarter of approximately \$31,000 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 2017 Quarter.

**Interest income.** Interest income increased to approximately \$763,000 for the 2018 Quarter from approximately \$121,000 for the 2017 Quarter. This increase was primarily due to higher investment cost basis and higher interest rates on investments.

**Interest expense.** Interest expense related to our loan and security agreement executed with Hercules in March 2017 was approximately \$862,000 for the 2018 Quarter compared to approximately \$110,000 for the 2017 Quarter. The increase was due to a higher principal balance and interest rates in the 2018 Quarter compared to the 2017 Quarter.

Comparison of the nine months ended September 30, 2018 and 2017



	Nine months ended September 30,			
	2018	2017	Change	% Change
Revenue:				
License revenue	\$ 25,000	\$ —	\$ 25,000	100%
Product revenue, net	508	—	508	100%
Total revenue	25,508	—	25,508	100%
Operating expenses:				
Costs of revenues, excluding amortization of acquired intangible assets	49	—	49	100%
Research and development	34,886	35,170	(284)	-1%
Selling, general and administrative	51,066	14,582	36,484	250%
Amortization of acquired intangible assets	31	—	31	100%
Total operating expenses	86,032	49,752	36,280	73%
Loss from operations	(60,524)	(49,752)	(10,772)	22%
Interest income	1,297	416	881	212%
Interest expense	(1,858)	(231)	(1,627)	704%
Net loss	\$ (61,085)	\$ (49,567)	\$ (11,518)	23%

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License revenue. Revenue for the nine months ended September 30, 2018 (2018 Period) was \$25.0 million and was related to upfront payments pursuant to the license and collaboration agreements executed between ourselves and Yakult and CSPC. We had no license revenue in the nine months ended September 30, 2017 (2017 Period).

Product revenue, net. We began commercial sales of COPIKTRA within the United States in September 2018, following receipt of FDA marketing approval on September 24, 2018. For the 2018 Period we recorded approximately \$508,000 of net product revenue. We had no product revenue during the 2017 Period.

Costs of revenues, excluding amortization of acquired intangible assets. Costs of revenues, excluding amortization of acquired intangible assets (cost of revenues) of approximately \$49,000 for the 2018 Period, consisted of costs associated with the manufacturing of COPIKTRA, royalties owed to 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 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 2018 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 expect cost of revenues to increase in relation to product revenues as we deplete these inventories. We had no cost of revenues during the 2017 Period.

Research and development expense. Research and development expense for the 2018 Period was \$34.9 million compared to \$35.2 million for the 2017 Period. The approximately \$284,000 decrease from the 2017 Period to the 2018 Period was primarily related to a decrease of \$6.0 million in license fees related to a one-time milestone payment pursuant to the Infinity license agreement that was recognized in the 2017 Period and a decrease of approximately \$975,000 in consulting fees, partially offset by increases of \$3.6 million in personnel related costs, including non-cash stock-based compensation, and \$2.7 million in CRO expense for outsourced biology, development and clinical services, which includes our clinical trial costs, and approximately \$443,000 in other costs.

We allocate the expenses related to external research and development services, such as CROs, clinical sites, manufacturing organizations and consultants by project. The table below summarizes our allocation of research and development expenses to our clinical programs, including COPIKTRA and defactinib, for the 2018 Period and the 2017 Period. 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. These unallocated research and development expenses are summarized in the table below and include approximate personnel related costs of \$6.9 million and \$3.9 million for the 2018 Period and the 2017 Period, respectively.

Nine months ended	
September 30,	
2018	2017
(in thousands)	

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COPIKTRA	\$ 20,187	\$ 23,125
Defactinib	1,691	2,326
Unallocated and other research and development expense	11,317	8,578
Unallocated stock-based compensation expense	1,691	1,141
Total research and development expense	\$ 34,886	\$ 35,170

Selling, general and administrative expense. Selling, general and administrative expense for the 2018 Period was \$51.1 million compared to \$14.6 million for the 2017 Period. The increase of \$36.5 million from the 2017 Period to the 2018 Period primarily resulted from an increase in consulting and professional fees of \$17.4 million, primarily related to the support of the commercial launch preparation activities, an increase in personnel related costs, including non-cash stock-based compensation, of \$16.1 million, primarily related to the hiring and staffing of our sales and commercial teams, and an increase in travel and other costs of \$3.0 million.

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Amortization of acquired intangible assets. Amortization of acquired intangible assets for the 2018 Period of approximately \$31,000 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 2017 Period.

Interest income. Interest income increased to approximately \$1.3 million for the 2018 Period from approximately \$416,000 for the 2017 Period. This increase was primarily due to higher investment cost basis and higher interest rates on investments.

Interest expense. Interest expense related to our loan and security agreement executed with Hercules in March 2017 was approximately \$1.9 million for the 2018 Period compared to approximately \$231,000 for the 2017 Period. The increase was due to a higher principal balance, higher interest rates, and an increase in the number of days outstanding in the 2018 Period compared to the 2017 Period.

## 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 5.00% Convertible Senior Notes due 2048 in October 2018. Following 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.

As of September 30, 2018, we had \$145.6 million in cash, cash equivalents and investments.

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 this Quarterly Report on Form 10-Q and in our Annual Report on Form 10-K for the year ended December 31, 2017 as filed with the Securities and Exchange Commission (SEC) on March 13, 2018 and in any subsequent filings with the SEC.

### Cash flows

The following table sets forth the primary sources and uses of cash for the 2018 Period and the 2017 Period (in thousands):

	Nine months ended September 30,	
	2018	2017
Net cash (used in) provided by:		
Operating activities	\$ (55,327)	\$ (36,983)
Investing activities	(11,574)	39,444
Financing activities	115,693	16,460
Increase in cash, cash equivalents and restricted cash	\$ 48,792	\$ 18,921

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.

Investing activities. The cash used in investing activities for the 2018 Period relates to the net purchases of investments of \$10.4 million and net purchases of property and equipment approximately \$1.2 million. The cash provided by investing activities for the 2017 Period reflects the net maturities of investments of \$39.4 million.

Financing activities. The cash provided by financing activities for the 2018 Period primarily represents \$81.2 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

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\$637,000 related to stock option exercises, offset by the payment of approximately \$324,000 of issuance costs related to a sale of our common stock during December 2017. The cash provided by financing activities for the 2017 Period primarily represents \$14.1 million in net proceeds received under our ATM, \$2.4 million in net proceeds received from our loan and security agreement executed with Hercules, and approximately \$91,000 received from the exercise of stock options, offset by approximately \$138,000 of deferred financing costs.

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 months ended September 30, 2018, there were no sales under the at-the-market equity offering program. During the nine months ended September 30, 2018, we sold 6,481,475 shares under this program for net proceeds of approximately \$24.3 million (after deducting commissions and other offering expenses). Through September 30, 2018, 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).

On May 16, 2018, we entered into an underwriting agreement with Cantor relating to the underwritten offering of 7,777,778 shares of our common stock (Underwriting Agreement). Cantor agreed to purchase the shares of our common stock pursuant to the Underwriting Agreement at a price of \$4.31 per share (Underwriting Agreement). In addition, we granted Cantor an option to purchase, at the public offering price less any underwriting discounts and commissions, an additional 1,166,666 shares of our common stock, exercisable for 30 days from the date of the prospectus supplement. The option was exercised by Cantor on May 23, 2018. The aggregate proceeds from Cantor, net of underwriting discounts and offering costs, were approximately \$38.3 million.

On June 14, 2018, we entered into a purchase agreement with Consonance Capital Master Account L.P. and P Consonance Opportunities Ltd. (collectively, Consonance) relating to the registered offering of 7,166,666 shares of our common stock at a price of \$6.00 per share (Purchase Agreement). The aggregate proceeds from Consonance, net of offering costs, were approximately \$42.8 million.

On October 17, 2018, we closed a registered direct public offering of \$150.0 million aggregate principal amount of our 5.00% Convertible Senior Notes due 2048 (the Notes), for net proceeds of \$145.1 million. The Notes are the senior unsecured obligations 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 our common stock, par value \$0.0001 per share (the Common Stock), 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. To the extent we have insufficient authorized but unissued shares to settle conversions in shares of Common Stock, we would be required to settle the deficiency in cash.

We will have the right, exercisable at our option, to cause all Notes then outstanding to be converted automatically if the “Daily VWAP” (as defined in the supplemental indenture governing the Notes) per share of the Common Stock equals or exceeds 130% of the conversion price on each of at least 20 trading days, whether or not consecutive, during any 30 day consecutive period commencing on or after the date the first Notes were issued.

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.

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License and collaboration agreements

Yakult

On June 5, 2018, we entered into a license and collaboration agreement (the Agreement) with Yakult, 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 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 Agreement. We retained all rights to duvelisib outside of Japan.

Yakult paid us an upfront, non-refundable payment of \$10.0 million in June 2018. We are 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 us 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 us in which Yakult has opted to participate (Global Clinical Trials) on a pro-rata basis.

Unless earlier terminated by either party, the 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 Agreement in its entirety at any time with 180 days' written notice. Either party may terminate the 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 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 Agreement. Either party may terminate the Agreement in its entirety upon certain insolvency events involving the other party.

We recognized the upfront payment of \$10.0 million as license revenue upon execution of the Agreement in June 2018.

CSPC



On July 26, 2018, we entered into an Exclusivity Agreement with CSPC which granted CSPC the exclusive right to negotiate a licensing agreement with us for duvelisib in China. CSPC paid us a non-refundable exclusivity fee of \$5.0 million in August 2018 (Exclusivity Fee) which was creditable against any payments agreed to under the terms of a potential definitive license agreement.

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 (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 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.

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As of September 30, 2018, CSPC is obligated to pay us an upfront, non-refundable payment of \$15.0 million, less the initial \$5.0 million Exclusivity Fee, resulting in an outstanding payment due of \$10.0 million, which is included in accounts receivable, net on the condensed consolidated balance sheets. The remaining \$10.0 million upfront payment was paid in full in November 2018. We are also 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 us 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 us in which CSPC has opted to participate (Global Clinical Trials) on a pro-rata basis.

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 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 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.

We recognized the upfront payment of \$15.0 million as license revenue upon execution of the CSPC Agreement during the three months ended September 30, 2018.

## Funding requirements

We expect to continue to incur significant expenses and operating losses for the foreseeable future. We anticipate that our expenses and operating losses will increase substantially if and 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.

We expect our existing cash, cash equivalents and investments will be sufficient to fund our obligations for at least the next twelve months from the date of filing of this Quarterly Report on Form 10-Q. We have based this estimate on assumptions that may prove to be wrong and we could use our available capital resources sooner than we currently expect. 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;

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- 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, 2017. There have not been any material changes from the contractual obligations and commitments previously disclosed in such report other than (i) a change in estimated obligations due to our landlord under the terms of our operating lease, entered into in April 2014, and amended effective February 2018, for our office space located in Needham, Massachusetts and (ii) our borrowing of an additional \$10.0 million from Hercules Capital, Inc. in June 2018. These changes are more fully described in Note 16, Commitments and contingencies and Note 9, Long-term debt, respectively, to our condensed consolidated financial statements appearing elsewhere 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 investments of \$145.6 million as of September 30, 2018, consisting of cash, U.S. Government money market funds, government-sponsored enterprise securities, and 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 September 30, 2018, an immaterial amount of our total liabilities was denominated in currencies other than the functional currency.

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As of September 30, 2018, we have borrowed \$25.0 million under the Amended Loan Agreement. The Amended Loan Agreement bears interest per annum equal to the greater of either (a) 10.5% or (b) the lesser of (i) 12.75% and (ii) the sum of (x) 10.5% plus (y) (A) the prime rate minus (B) 4.5%. 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 nine months ended September 30, 2018.

### Item 4. Controls and Procedures.

#### Evaluation of disclosure controls and procedures

Our management, with the participation of our Chief Executive Officer and our Chief Financial Officer, evaluated the effectiveness of our disclosure controls and procedures as of September 30, 2018. The term “disclosure controls and procedures,” as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act 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 September 30, 2018, our Chief Executive Officer and our Chief 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

During the quarter ended September 30, 2018, we began generating product revenue from the sale of COPIKTRA in the United States. We consider the accounting for our net product revenue to be material to the results of operations for the three and nine months ended September 30, 2018, and believe that the additional internal controls and procedures relating to the accounting for net product revenues, as well as adoption of ASC Topic 606, Revenue from Contracts with Customers in connection therewith, and related commercial inventory, have a material effect on our internal control over financial reporting. During the three and nine months ended September 30, 2018, there were no further changes in our internal controls over financial reporting. See Note 2, Summary of significant accounting policies, to our unaudited condensed consolidated financial statements contained in this Quarterly Report on Form 10-Q for further details.

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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, 2017 as filed with the SEC on March 13, 2018. 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, 2017, except as noted below.

Risks Related to the Commercialization of COPIKTRA and Development of Our Product Candidates

We are dependent on the commercial success of COPIKTRA.

A majority of our time, resources and effort are focused on the commercialization of COPIKTRA in the United States. While we expect to continue to expend significant time, resources and effort on the development of our other product candidates, they are in earlier stages of development and subject to the risks of failure inherent in developing drug products.

Our ability to successfully commercialize COPIKTRA will depend on, among other things, our ability to:

- maintain commercial manufacturing arrangements with third-party manufacturers;
- produce, through a validated process, sufficiently large quantities and inventory of COPIKTRA to meet demand;
- build and maintain internal sales, distribution and marketing capabilities sufficient to generate commercial sales of COPIKTRA;
- secure widespread acceptance of our product from physicians, health care payors, patients and the medical community;
- properly price and obtain coverage and adequate reimbursement of COPIKTRA by governmental authorities, private health insurers, managed care organizations and other third-party payors;
- maintain compliance with ongoing FDA labeling, packaging, storage, advertising, promotion, recordkeeping, safety and other post-market requirements;
- manage our growth and spending as costs and expenses increase due to commercialization; and
- establish and maintain collaborations with third parties for the commercialization of COPIKTRA in countries outside the United States, and such collaborators' ability to obtain regulatory and reimbursement approvals in such countries.

There are no guarantees that we will be successful in completing these tasks. In addition, we have begun, and will need to continue investing substantial financial and management resources to build out our commercial infrastructure

and to recruit and train sufficient additional qualified marketing, sales and other personnel in support of our sales of COPIKTRA.

Sales of COPIKTRA may be slow or limited for a variety of reasons including competing therapies or safety issues. If COPIKTRA is not successful in gaining broad commercial acceptance, our business would be harmed.

Any sales of COPIKTRA will be dependent on several factors including our ability to educate and increase physician awareness of the benefits and cost-effectiveness of COPIKTRA relative to competing therapies. The degree of market acceptance of COPIKTRA among physicians, patients, health care payors and the medical community will depend on a number of factors, including:



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- acceptable evidence of safety and efficacy;
- relative convenience and ease of administration;
- prevalence and severity of any adverse side effects;
- availability of alternative treatments;
- pricing and cost effectiveness;
- effectiveness of our sales and marketing capability and strategies;
- ability to obtain sufficient third-party coverage and reimbursement;
- changes in the standard of care for the targeted indications for COPIKTRA;
  - warnings and limitations, including the boxed warning related to the risks of infections, diarrhea or colitis, cutaneous reactions, and pneumonitis, contained in the approved labeling for COPIKTRA;
- safety concerns with similar products marketed by others;
- the prevalence and severity of any side effects as a result of treatment with COPIKTRA;
- our ability to comply with FDA post-marketing requirements imposed upon COPIKTRA, including conducting and completing a confirmatory clinical trial in patients with relapsed or refractory follicular lymphoma that verifies and isolates the benefits of COPIKTRA; and
- the actual market-size for COPIKTRA, which may be larger or smaller than expected.

In addition, COPIKTRA will be subject to continual review by the FDA, and we cannot assure you that newly discovered or developed safety issues will not arise. With the use of any newly marketed drug by a wider patient population, serious adverse events may occur from time to time that initially do not appear to relate to the drug itself. Any safety issues could cause us to suspend or cease marketing COPIKTRA, cause us to modify how we market COPIKTRA, subject us to substantial liabilities and adversely affect our revenues and financial condition. In the event of a withdrawal of COPIKTRA from the market, our revenues would decline significantly and our business would be seriously harmed and could fail. We additionally may experience significant fluctuations in sales of COPIKTRA from period to period and, ultimately, we may never generate sufficient revenues from COPIKTRA to reach or maintain profitability or sustain our anticipated operations.

Preclinical testing and clinical trials of our product candidates may not be successful. In the near term, we are dependent on the success of our PI3K inhibitor program, including COPIKTRA. If we are unable to obtain marketing approval for or successfully commercialize any of our other product candidates, or if we experience significant delays in doing so, our business will be materially harmed.

We have invested a significant portion of our efforts and financial resources in the research and development of our product candidates, including COPIKTRA, for which we are conducting clinical trials in multiple indications. We received FDA approval for COPIKTRA 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 were granted accelerated approval of COPIKTRA for the treatment of adult patients with relapsed or refractory follicular lymphoma (FL) after at least two prior systemic therapies. Our ability to generate product revenues will depend heavily on the successful commercialization of COPIKTRA and development of our other product candidates. The success of our product candidates will depend on several factors, including the following:

- initiation and successful enrollment and completion of our clinical trials;
- receipt of marketing approvals from the FDA and other regulatory authorities for our future product candidates, including pricing approvals where required;
- establishing and maintaining commercial manufacturing capabilities or making arrangements with third-party manufacturers;
- obtaining and maintaining patent and trade secret protection and regulatory exclusivity for our product candidates;
- establishing and maintaining commercial capabilities, including hiring and training a sales force, and launching commercial sales of the products, if and when approved, whether alone or in collaboration with others;



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- acceptance of the products, if and when approved, by patients, the medical community and third-party payors;
  - securing and maintaining coverage and adequate reimbursement for our products from third party payors;
- effectively competing with other therapies; and
- a continued acceptable safety and efficacy profile of the products following approval.

Many of these factors are beyond our control, including clinical development, the regulatory submission process, potential threats to our intellectual property rights and the manufacturing, marketing and sales efforts of any collaborator. If we do not achieve one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize our product candidates, which would materially harm our business.

If clinical trials of our product candidates fail to demonstrate safety and efficacy to the satisfaction of regulatory authorities or do not otherwise produce positive results, we may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of our product candidates.

Before obtaining marketing approval from regulatory authorities for the sale of our product candidates, we must complete extensive clinical trials to demonstrate the safety and efficacy of our product candidates in humans. Clinical testing is expensive, difficult to design and implement, can take many years to complete and is uncertain as to outcome. A failure of one or more clinical trials can occur at any stage of testing. The outcome of preclinical testing and early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results. For example, a further review and analysis of this data may change the conclusions drawn from this unaudited data indicating less promising results than we currently anticipate.

In some instances, there can be significant variability in safety and/or efficacy results between different trials of the same product candidate due to numerous factors, including changes in trial protocols, differences in size and type of the patient populations, adherence to the dosing regimen and other trial protocols and the rate of dropout among clinical trial participants. There also may be significant variability in the safety results obtained through the long-term follow-up of patients from ongoing studies. We do not know whether any clinical trial we may conduct or follow-up data we collect will demonstrate consistent or adequate efficacy and/or safety sufficient to obtain regulatory approval to market our product candidates.

In addition, the design of a clinical trial may determine whether its results will support approval of a product and flaws in the design of a clinical trial may not become apparent until the clinical trial is well advanced. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their products.

A failure of one or more clinical trials could indicate a higher likelihood that subsequent clinical trials of the same product candidate in the same or other indications or subsequent clinical trials of other related product candidates will be unsuccessful for the same reasons as the unsuccessful clinical trials.

We may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent our ability to receive marketing approval or commercialize our product candidates, including:

- regulators or institutional review boards may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- we may have delays in reaching or fail to reach agreement on clinical trial contracts or clinical trial protocols with prospective trial sites;
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clinical trials of our product candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon product development programs;

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- the number of patients required for clinical trials of our product candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate our participants may drop out of these clinical trials at a higher rate than we anticipate;
- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- regulators or institutional review boards may require that we or our investigators suspend or terminate clinical trials for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks;
- the cost of clinical trials of our product candidates may be greater than we anticipate;
  - the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate; and
- our product candidates may have undesirable side effects or other unexpected characteristics, causing us or our investigators, regulators or institutional review boards to suspend or terminate the trials.

If we are required to conduct additional clinical trials or other testing of our product candidates beyond those that we currently contemplate, if we are unable to successfully complete clinical trials of our product candidates or other testing, if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we may:

- be delayed in obtaining or not obtain marketing approval for our product candidates;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions including imposition of a Risk Evaluation and Mitigation Strategy (REMS), or safety warnings, including boxed warnings;
- be subject to additional post marketing testing requirements; or
- have the product removed from the market after obtaining marketing approval.

The FDA and foreign regulatory authorities may determine that the results from our ongoing and future trials do not support regulatory approval and may require us to conduct an additional clinical trial or trials. If these agencies take such a position, the costs of development of our product candidates could increase materially and their potential market introduction could be delayed. The regulatory agencies could also require that we conduct additional clinical, nonclinical or manufacturing validation studies and submit that data before it will consider an NDA. Our product development costs will also increase if we experience delays in clinical testing or marketing approvals. We do not know whether any clinical trials will begin as planned, will need to be restructured or will be completed on schedule, or at all. Significant clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do and impair our ability to successfully commercialize our product candidates and may harm our business and results of operations.

If we experience delays or difficulties in the enrollment of patients in clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented.

We may not be able to initiate or continue clinical trials for our product candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA or similar regulatory authorities outside the United States. In addition, there are a number of ongoing clinical trials being conducted by other companies for product candidates treating cancer. Patients who would otherwise be eligible for our clinical trials may instead enroll in clinical trials of our competitors' product candidates, particularly if they view such treatments to be more conventional and established.

Patient enrollment is affected by other factors including:

- the size and nature of the patient population;

- severity of the disease under investigation;
- eligibility criteria for the study in question;

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- perceived risks and benefits of the product candidate under study in relation to other available treatments including any new treatments that may be approved for the indications we are investigating;
- efforts to facilitate timely enrollment in clinical trials;
- patient referral practices of physicians;
- the ability to monitor patients adequately during and after treatment; and
- proximity and availability of clinical trial sites for prospective patients.

Furthermore, enrolled patients may drop out of a clinical trial, which could impair the validity or statistical significance of the clinical trial. A number of factors can influence the patient discontinuation rate, including, but not limited to:

- the inclusion of a placebo arm in a trial;
- possible inactivity or low activity of the product candidate being tested at one or more of the dose levels being tested;
- the occurrence of adverse side effects, whether or not related to the product candidate; and
- the availability of numerous alternative treatment options, including clinical trials evaluating competing product candidates, that may induce patients to discontinue their participation in the trial.

Our inability to enroll a sufficient number of patients for our clinical trials would result in significant delays or may require us to abandon one or more clinical trials altogether. Enrollment delays in our clinical trials may result in increased development costs for our product candidates, which would cause the value of our company to decline and limit our ability to obtain additional financing.

If serious adverse or unexpected side effects are identified during the commercialization of COPIKTRA or development of our other product candidates, we may need to abandon or limit the commercialization of COPIKTRA and abandon or limit our development of some of our other product candidates.

The FDA approved COPIKTRA with labeling that includes a boxed warning for four fatal and/or serious toxicities: infections, diarrhea or colitis, cutaneous reactions, and pneumonitis. As a requirement of the FDA's approval, we are implementing an informational REMS to provide appropriate dosing and safety information to better support physicians in managing their patients on COPIKTRA. In addition to the boxed warning, use of COPIKTRA is also associated with adverse reactions, which may require dose reduction, treatment delay or discontinuation of COPIKTRA. Warnings and precautions are provided for infections, diarrhea or colitis, cutaneous reactions, pneumonitis, hepatotoxicity, neutropenia, and embryo-fetal toxicity. The most common adverse reactions (reported in  $\geq 20\%$  of patients) were diarrhea or colitis, neutropenia, rash, fatigue, pyrexia, cough, nausea, upper respiratory infection, pneumonia, musculoskeletal pain, and anemia.

Our other product candidates are in various stages of clinical development and their risk of failure is high. It is impossible to predict when or if our other product candidates will prove effective or safe in humans or will receive marketing approval. If our product candidates are associated with undesirable side effects or have characteristics that are unexpected, we may need to abandon their development or limit development to certain uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk benefit perspective. Patients in our clinical trials have experienced serious adverse events, deemed by us and the clinical investigator to be related to our product candidates. Serious adverse events generally refer to adverse events, that result in death, are life threatening, require hospitalization or prolonging of hospitalization, or cause a significant and permanent disruption of normal life functions, congenital anomalies or birth defects, or require intervention to prevent such outcomes.

Defactinib is in our Phase 1 and Phase 2 clinical trials and the development program continues to progress. The toxicities reported thus far are consistent with other drugs in this class.





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As a result of adverse events observed to date, or further safety or toxicity issues that we may experience in our clinical trials in the future, we may not receive approval to market any product candidates, which could prevent us from ever generating revenue from the sale of products or achieving profitability. Results of our trials could reveal an unacceptably high severity and prevalence of side effects. In such an event, our trials could be suspended or terminated and the FDA or comparable foreign regulatory authorities could order us to cease further development of or deny approval of our products candidates for any or all targeted indications. Many compounds that initially showed promise in early stage testing for treating cancer have later been found to cause side effects that prevented further development of the compound. In addition, while we and our clinical trial investigators currently determine if serious adverse or unacceptable side effects are drug related, the FDA or other non-U.S. regulatory authorities may disagree with our or our clinical trial investigators' interpretation of data from clinical trials and the conclusion that a serious adverse effect or unacceptable side effect was not drug related.

For COPIKTRA, if we or others identify previously unknown side effects or if known side effects are more frequent or severe than in the past, then:

- sales of COPIKTRA may be modest;
- regulatory approvals for COPIKTRA may be restricted or withdrawn;
- we may decide to, or be required to, send product warning letters or field alerts to physicians, pharmacists and hospitals;
- additional non-clinical or clinical studies, changes in labeling or changes to manufacturing processes, specifications and/or facilities may be required; and
- government investigations or lawsuits, including class action suits, may be brought against us.

Any of the above occurrences would harm or prevent sales of COPIKTRA, increase our expenses and impair our ability to successfully commercialize COPIKTRA. Furthermore, as COPIKTRA is commercially available, it may be used in a wider population and in a less rigorously controlled environment than in clinical studies. As a result, regulatory authorities, healthcare practitioners, third-party payors or patients may perceive or conclude that the use of COPIKTRA is associated with previously unknown serious adverse effects, undermining our commercialization efforts.

Preclinical studies and preliminary and interim data from clinical trials of our product candidates are not necessarily predictive of the results or success of ongoing or later clinical trials of our product candidates. If we cannot replicate the results from our preclinical studies and clinical trials of our product candidates, we may be unable to successfully develop, obtain regulatory approval for and commercialize our product candidates.

Preclinical studies and any positive preliminary and interim data from our clinical trials of our product candidates may not necessarily be predictive of the results of ongoing or later clinical trials. Even if we are able to complete our planned clinical trials of our product candidates according to our current development timeline, the positive results from clinical trials of our product candidates may not be replicated in subsequent clinical trial results. Also, our later stage clinical trials could differ in significant ways from earlier stage clinical trials, which could cause the outcome of the later stage trials to differ from our earlier stage clinical trials. For example, these differences may include changes to inclusion and exclusion criteria, efficacy endpoints and statistical design. Many companies in the pharmaceutical and biotechnology industries, including us, have suffered significant setbacks in late stage clinical trials after achieving positive results in an earlier stage of development. If we fail to produce positive results in our planned clinical trials of any of our product candidates, the development timeline and regulatory approval and commercialization prospects for our product candidates, and, correspondingly, our business and financial prospects, would be materially adversely affected.

Our approach to the treatment of cancer through the killing of cancer cells and disruption of the tumor microenvironment is relatively unproven, and we do not know whether we will be able to develop any products of

significant commercial value.

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We are commercializing COPIKTRA and developing duvelisib in other indications and other product candidates to treat cancer by using targeted agents to kill cancer cells or disrupt the tumor microenvironment and thereby thwart their growth and proliferation of cancer cells.

Research on the use of small molecules to inhibit PI3K and FAK signaling pathways and disrupt the tumor microenvironment is an emerging field and, consequently, there is still uncertainty about whether COPIKTRA and defactinib are effective in improving outcomes for patients with cancer. With respect to our FAK inhibition program, there is some debate in the scientific community regarding cancer stem cells (CSCs), the existence of these cells, the defining characteristics of these cells, as well as whether targeting such cells is an effective approach to treating cancer. Some believe that targeting CSCs as part of our multi-faceted approach should be sufficient for a positive clinical outcome, while others believe that, at times or always, the use of FAK inhibitors that reduce CSCs should be coupled with conventional chemotherapies for a positive clinical outcome.

Any products that we develop may not effectively target cancer cells, enhance anti-tumor immunity, or modulate the local tumor microenvironment. While we are currently commercializing COPIKTRA and conducting clinical trials for other product candidates that we believe will attack cancer cells through the inhibition of the PI3K or FAK signaling pathways and potentially disrupt the tumor microenvironment, we may not ultimately be successful in demonstrating their efficacy, alone or in combination with other treatments.

The approval of our product candidates as part of a combination therapy for the treatment of certain cancers may be more costly than our prior clinical trials, may take longer to achieve regulatory approval, may be associated with new, more severe or serious and unanticipated adverse events, and may have a smaller market opportunity.

Part of our current business model involves conducting clinical trials to study the effects of combining our product candidates with other approved and investigational targeted therapies, chemotherapies, and immunotherapies to treat patients with cancer. Regulatory approval for a combination treatment generally requires clinical trials to evaluate the activity of each component of the combination treatment. As a result, it may be more difficult and costly to obtain regulatory approval of our product candidates for use as part of a combination treatment than obtaining regulatory approval of our product candidates alone. In addition, we also risk losing the supply of any approved or investigational product being combined with our product candidate in these clinical trials. Furthermore, the potential market opportunity for our product candidates is difficult to estimate precisely. For instance, if one of our product candidates receives regulatory approval from a combination study, it may be approved solely for use in combination with the approved or investigational product in a particular indication and the market opportunity our product candidate would be dependent upon the continued use and availability of the approved or investigational product. In addition, because physicians, patients and third-party payors may be sensitive to the addition of the cost of our product candidates to the cost of treatment with the other products, we may experience downward pressure on the price that we can charge for our product candidates if they receive regulatory approval. Further, we cannot be sure that physicians will view our product candidates, if approved as part of a combination treatment, as sufficiently superior to a treatment regimen consisting of only the approved or investigational product. Additionally, the adverse side effects of our product candidates may be enhanced when combined with other products. If such adverse side effects are experienced, we could be required to conduct additional pre-clinical and clinical studies and if such adverse side effects are severe, we may not be able to continue the clinical trials of the combination therapy because the risks may outweigh the therapeutic benefit of the combination.

We may not be successful in obtaining necessary rights to compounds and product candidates for our development pipeline through acquisitions and in-licenses.

We may seek to acquire new compounds and product candidates from other pharmaceutical and biotechnology companies, academic scientists and other researchers, such as our exclusive in-license from Infinity

Pharmaceuticals, Inc. (Infinity), to research, develop, commercialize, and manufacture products in oncology indications containing duvelisib. The success of this strategy depends partly upon our ability to identify, select, discover and acquire promising pharmaceutical product candidates and products. The process of proposing, negotiating and implementing a license or acquisition of a product candidate or approved product is lengthy and complex. Other companies, including

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some with substantially greater financial, marketing and sales resources, may compete with us for the license or acquisition of product candidates and approved products. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We have limited resources to identify and execute the acquisition or in-licensing of third-party products, businesses and technologies and integrate them into our current infrastructure. Moreover, we may devote resources to potential acquisitions or in-licensing opportunities that are never completed, or we may fail to realize the anticipated benefits of such efforts. We also may be unable to license or acquire the relevant compound or product candidate on terms that would allow us to make an appropriate return on our investment. Any product candidate that we acquire may require additional development efforts prior to commercial sale, including manufacturing, pre-clinical testing, extensive clinical testing and approval by the FDA and applicable foreign regulatory authorities. All product candidates are prone to risks of failure typical of pharmaceutical product development.

In addition, future product or business acquisitions may entail numerous operational and financial risks, including:

- exposure to unknown liabilities;
- disruption of our business and diversion of our management's time and attention to develop acquired products, product candidates or technologies;
- higher than expected acquisition and integration costs;
- increased amortization expenses; and
- incurrence of substantial debt, dilutive issuances of securities or depletion of cash to pay for acquisitions.

Future business acquisitions may also entail certain additional risks, such as:

- difficulty in combining the operations and personnel of any acquired businesses with our operations and personnel;
- impairment of relationships with key suppliers or customers of any acquired businesses due to changes in management and ownership; and
- inability to motivate key employees of any acquired businesses.

If we fail to obtain regulatory approval in jurisdictions outside the United States, we will not be able to market our products in those jurisdictions.

We intend to seek regulatory approval for our product candidates, including COPIKTRA, in a number of countries outside of the United States and expect that these countries will be important markets for our products, if approved. Marketing our products in these countries will require separate regulatory approvals in each market and compliance with numerous and varying regulatory requirements. The regulations that apply to the conduct of clinical trials and approval procedures vary from country to country and may require additional testing. Moreover, the time required to obtain approval may differ from that required to obtain FDA approval. In addition, in many countries outside the United States, a drug must be approved for reimbursement before it can be approved for sale in that country. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one foreign regulatory authority does not ensure approval by regulatory authorities in other foreign countries or by the FDA. Failure to obtain regulatory approval in one country may have a negative effect on the regulatory approval process in others. The foreign regulatory approval process may include all of the risks associated with obtaining FDA approval. We may not obtain foreign regulatory approvals on a timely basis, if at all. We may not be able to file for regulatory approvals and may not receive necessary approvals to commercialize our products in any foreign market.

We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and managerial resources, we focus on research programs and product candidates that we identify for specific indications. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential. Our resource



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allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable products.

We face substantial competition, which may result in others developing or commercializing products before or more successfully than we do.

The development and commercialization of new drug products is highly competitive. We face competition with respect to COPIKTRA and our other product candidates and will face competition with respect to any product candidates that we may seek to develop or commercialize in the future, from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. There are a number of large pharmaceutical and biotechnology companies that currently market and sell products or are pursuing the development of products for the treatment of the disease indications for which we are developing COPIKTRA and our product candidates, including Gilead Sciences, Inc., Abbvie, Pharmacyclics LLC, Roche, Celgene Corporation, AstraZeneca, Incyte Corporation, TG Therapeutics, Inc., Novartis and others. Some of these competitive products and therapies are based on scientific approaches that are the same as or similar to our approach, and others are based on entirely different approaches. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization.

We are commercializing COPIKTRA and developing our other product candidates for the treatment of cancer. There are a variety of available therapies marketed for cancer. In many cases, these drugs are administered in combination to enhance efficacy. Some of these drugs are branded and subject to patent protection, and others are available on a generic basis. Many of these approved drugs are well established therapies and are widely accepted by physicians, patients and third-party payors. Insurers and other third-party payors may also encourage the use of generic products. We expect that COPIKTRA and our other product candidates, if approved, will be priced at a significant premium over competitive generic products.

Many of our competitors have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller and other early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

In addition, to the extent that products or product candidates of our competitors demonstrate serious adverse side effects or are determined to be ineffective in clinical trials, the commercialization of COPIKTRA and the development of our other product candidates could be negatively impacted.

COPIKTRA and any future product candidates that we commercialize may become subject to unfavorable pricing regulations or third-party coverage and reimbursement policies, which would harm our business.

In both domestic and foreign markets, sales of COPIKTRA and any product candidates that may receive marketing approval in the future will depend, in part, on favorable pricing as well as the availability of coverage and amount of reimbursement by third party payors, including governments and private health plans. Substantial uncertainty exists regarding coverage and reimbursement by third party payors of newly approved health care products.

Outside the United States, some countries require approval of the sale price of a drug before the product can be marketed. In many such countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a product in a particular



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country, but then be subject to price regulations that delay our commercial launch of the product, possibly for lengthy time periods, and negatively impact the revenues we are able to generate from the sale of the product in that country. Adverse pricing limitations may hinder our ability to recoup our investment in COPIKTRA and other product candidates, even if those product candidates obtain marketing approval.

Cost containment is a key trend in the United States and elsewhere. Third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. We cannot be sure that coverage and reimbursement will be available for COPIKTRA or any other product that we commercialize and, if reimbursement is available, the level of reimbursement. Coverage and reimbursement may impact the demand for, or the price of, COPIKTRA or any other product candidate for which we obtain marketing approval. If coverage and reimbursement are not available or reimbursement is available only to limited levels, we may not be able to successfully commercialize COPIKTRA or any other product candidate for which we may obtain marketing approval.

If we participate in and then fail to comply with our reporting and payment obligations under the Medicaid Drug Rebate Program or other governmental pricing programs in the U.S., we could be subject to additional reimbursement requirements, penalties, sanctions and fines which could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

With the approval of COPIKTRA, we anticipate that we will need to participate in the Medicaid Drug Rebate Program, Medicare Coverage Gap Discount Program and a number of other federal and state government pricing programs in the U.S. in order to obtain coverage for the product by certain government healthcare programs. These programs would generally require us to pay rebates or provide discounts to certain private purchasers or government payors in connection with our products when dispensed to beneficiaries of these programs. In some cases, such as with the Medicaid Drug Rebate Program, the rebates are based on pricing and rebate calculations that we report on a monthly and quarterly basis to the government agencies that administer the programs. The terms, scope and complexity of these government pricing programs change frequently. We may also have reimbursement obligations or be subject to penalties if we fail to provide timely and accurate information to the government, pay the correct rebates or offer the correct discounted pricing. Changes to the price reporting or rebate requirements of these programs would affect our obligations to pay rebates or offer discounts. Responding to current and future changes may increase our costs and the complexity of compliance, will be time-consuming, and could have a material adverse effect on our results of operations.

Product liability lawsuits against us could cause us to incur substantial liabilities and to limit commercialization of any products that we may develop, including COPIKTRA.

We face an inherent risk of product liability exposure related to the testing of our product candidates in human clinical trials and will face an even greater risk from any sales of COPIKTRA or if we commercially sell any other products we may develop. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for COPIKTRA or any other product candidates or products that we may develop;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend the related litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue; and

- the inability to commercialize any products that we may develop.

We currently hold \$10.0 million in product liability insurance coverage in the aggregate, with a per incident limit of \$10.0 million, which may not be adequate to cover all liabilities that we may incur. We may need to increase our

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insurance coverage as we commercialize COPIKTRA and any future product candidates or if we initiate additional clinical trials in the United States and around the world. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties.

Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of biological, hazardous or radioactive materials.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

### Risks Related to Our License Agreement with Infinity

If we do not realize the anticipated benefits of our license agreement with Infinity for the COPIKTRA program, our business could be adversely affected.

Our license agreement with Infinity for COPIKTRA may fail to further our business strategy as anticipated or to achieve anticipated benefits and success. We may make or have made assumptions relating to the impact of the acquisition of COPIKTRA on our financial results relating to numerous matters, including:

- the cost of development and commercialization of COPIKTRA; and
- other financial and strategic risks related to the license agreement with Infinity.

Further, we may incur higher than expected operating and transaction costs, and we may encounter general economic and business conditions that adversely affect us relating to our license agreement with Infinity. If one or more of these assumptions are incorrect, it could have an adverse effect on our business and operating results, and the benefits from our license agreement with Infinity for COPIKTRA may not be realized or be of the magnitude expected.

### Risks Related to Our Financial Position and Need for Additional Capital

We have incurred significant losses since our inception. We expect to incur losses for the foreseeable future and may never achieve or maintain profitability.

Since inception, we have incurred significant operating losses. As of September 30, 2018, we had an accumulated deficit of \$364.2 million. To date, we have generated minimal product revenues and have financed our operations primarily through public offerings of our common stock, sales of our common stock pursuant to our at-the-market equity offering programs, and our loan and security agreement with Hercules Capital Inc. (Hercules). As of

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September 30, 2018, there was \$25.0 million available to borrow under the amended term loan facility with Hercules, subject to certain conditions of funding. We have devoted substantially all of our efforts to research and development. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. The net losses we incur may fluctuate significantly from quarter to quarter. We anticipate that our expenses will increase substantially if and as we:

- commercialize COPIKTRA;
- continue our ongoing clinical trials with our product candidates, 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 obtain marketing approval.

To become and remain profitable, we must develop and eventually commercialize a product or products with significant market potential, such as COPIKTRA. This will require us to be successful in a range of challenging activities, including completing preclinical testing and clinical trials of our product candidates, obtaining marketing approval for these product candidates and manufacturing, marketing and selling those products for which we may obtain marketing approval. We may never succeed in these activities and, even if we do, may never generate revenues that are significant or large enough to achieve profitability. If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of the company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations. A decline in the value of our company could also cause you to lose all or part of your investment.

We will continue to need substantial additional funding. If we are unable to raise capital when needed, we would be forced to delay, reduce or eliminate our product development programs or commercialization efforts, including for COPIKTRA.

We expect our expenses to increase in connection with our ongoing activities, particularly as we commercialize COPIKTRA and continue the clinical development of our other product candidates. We expect to incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution of COPIKTRA. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations, including for our clinical development programs and any commercialization efforts for COPIKTRA.

We expect our cash, cash equivalents and investments at September 30, 2018 will be sufficient to fund our current operating plan and capital expenditure requirements beyond the next twelve months. 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;



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- 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.

Conducting clinical trials is a time consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain marketing approval of any of our other product candidates. Even though the FDA approved COPIKTRA, it may not achieve commercial success. Our commercial revenues will be derived from sales of products, such as COPIKTRA. Accordingly, even though we received regulatory approval for COPIKTRA, it will take several years to achieve peak sales, and we will need to continue to rely on additional financing to further our clinical development objectives. Adequate additional financing may not be available to us on acceptable terms, or at all.

Raising additional capital or entering into certain licensing arrangements may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to COPIKTRA and our other product candidates.

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, grants and government funding, strategic alliances and licensing arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt, 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. To the extent that we enter into certain licensing arrangements, the ownership interest of our existing stockholders may be diluted if we elect to make certain payments in shares of our common stock. For example, pursuant to the terms of our license agreement with Infinity, we may elect to make certain milestone payments in shares of common stock in lieu of cash, according to a formula set forth in the license agreement. 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. For example, see our risk factors under the heading "Risks Related to Our Indebtedness."

If we raise additional funds through collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish future revenue streams or valuable rights to COPIKTRA or other product candidates or to 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 COPIKTRA and other product candidates that we would otherwise prefer to develop and market ourselves.

### Risks Related to Our Indebtedness

Our level of indebtedness and debt service obligations could adversely affect our financial condition, and may make it more difficult for us to fund our operations.

In March 2017, we entered into a Loan and Security Agreement with Hercules, which was subsequently amended in January, March and October 2018. Under the Loan and Security Agreement, as amended (the Amended Loan Agreement), Hercules will provide access to term loans with an aggregate principal amount of up to \$50.0 million. As of September 30, 2018, there was \$25.0 million available to borrow under the Amended Loan Agreement, subject to certain conditions of financing.

All obligations under the Amended Loan Agreement are secured by substantially all of our existing property and assets, excluding our intellectual property. This indebtedness may create additional financing risk for us, particularly if our business or prevailing financial market conditions are not conducive to paying off or refinancing our outstanding debt obligations at maturity. This indebtedness could also have important negative consequences,

including:

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- we will need to repay our indebtedness by making payments of interest and principal, which will reduce the amount of money available to finance our operations, our research and development efforts and other general corporate activities; and
- our failure to comply with the restrictive covenants in the Amended Loan Agreement could result in an event of default that, if not cured or waived, would accelerate our obligation to repay this indebtedness, and Hercules could seek to enforce their security interest in the assets securing such indebtedness.

To the extent additional debt is added to our current debt levels, the risks described above could increase.

We may not have cash available in an amount sufficient to enable us to make interest or principal payments on our indebtedness when due.

Failure to satisfy our current and future debt obligations under the Amended Loan Agreement, or breaching any covenants under the Amended Loan Agreement, subject to specified cure periods with respect to certain breaches, could result in an event of default and, as a result, Hercules could accelerate all of the amounts due. In the event of an acceleration of amounts due under the Amended Loan Agreement as a result of an event of default, we may not have enough available cash or be able to raise additional funds through equity or debt financings to repay such indebtedness at the time of such acceleration. In that case, we may be required to delay, limit, reduce or terminate our COPIKTRA commercialization efforts, other product candidate development or grant to others the rights to develop and market COPIKTRA and our other product candidates that we would otherwise prefer to develop and market internally. Hercules could also exercise its rights as collateral agent to take possession and dispose of the collateral securing the term loans for its benefit, which collateral includes substantially all of our property other than our intellectual property. Our business, financial condition and results of operations could be materially adversely affected as a result of any of these events. We are subject to certain restrictive covenants which, if breached, could have a material adverse effect on our business and prospects.

The Amended Loan Agreement imposes operating and other restrictions on us. Such restrictions will affect, and in many respects limit or prohibit, our ability and the ability of any future subsidiary to, among other things:

- dispose of certain assets;
- change our lines of business;
- engage in mergers, acquisitions or consolidations;
- incur additional indebtedness;
- create liens on assets;
- pay dividends and make distributions or repurchase our capital stock; and
- engage in certain transactions with affiliates.

### Risks Related to Our Dependence on Third Parties

We rely in part on third parties to conduct our clinical trials and preclinical testing, and if they do not properly and successfully perform their obligations to us, we may not be able to commercialize COPIKTRA or obtain regulatory approvals for and commercialize any of our other product candidates.

We rely on third parties, such as contract research organizations (CROs), clinical data management organizations, medical institutions and clinical investigators, to conduct, provide monitors for and manage data from all of our clinical trials. We compete with many other companies for the resources of these third parties.

Any of these third parties may terminate their engagements with us at any time. If we need to enter into alternative arrangements, it would delay our product development activities and ultimately the commercialization of our product candidates.



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Our reliance on these third parties for research and development activities will reduce our control over these activities but will not relieve us of our responsibilities. For example, we will remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA and other regulatory agencies require us to comply with standards, commonly referred to as Good Clinical Practices (GCP) for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. Regulatory authorities enforce these GCP requirements through periodic inspections of trial sponsors, principal investigators and trial sites. If we or any of our CROs fail to comply with applicable GCP requirements, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or other regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot assure you that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical trials comply with GCP requirements. We also are required to register ongoing clinical trials and post the results of completed clinical trials on government-sponsored databases, such as ClinicalTrials.gov, within certain timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions.

If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we will not be able to obtain, or may be delayed in obtaining, marketing approvals for some of our product candidates and will not be able to, or may be delayed in our efforts to, successfully commercialize COPIKTRA and our other product candidates.

We intend to rely on third parties to conduct investigator sponsored clinical trials of our product candidates. Any failure by a third party to meet its obligations with respect to the clinical development of our product candidates may delay or impair our ability to obtain regulatory approval for our product candidates.

We intend to rely on academic and private non-academic institutions to conduct and sponsor clinical trials relating to our product candidates. We will not control the design or conduct of the investigator sponsored trials, and it is possible that the FDA or non-U.S. regulatory authorities will not view these investigator-sponsored trials as providing adequate support for future clinical trials, whether controlled by us or third parties, for any one or more reasons, including elements of the design or execution of the trials or safety concerns or other trial results.

Such arrangements will provide us certain information rights with respect to the investigator sponsored trials, including access to and the ability to use and reference the data, including for our own regulatory filings, resulting from the investigator sponsored trials. However, we do not have control over the timing and reporting of the data from investigator sponsored trials, nor do we own the data from the investigator sponsored trials. If we are unable to confirm or replicate the results from the investigator sponsored trials or if negative results are obtained, we would likely be further delayed or prevented from advancing further clinical development of our product candidates. Further, if investigators or institutions breach their obligations with respect to the clinical development of our product candidates, or if the data proves to be inadequate compared to the firsthand knowledge we might have gained had the investigator sponsored trials been sponsored and conducted by us, then our ability to design and conduct any future clinical trials ourselves may be adversely affected.

Additionally, the FDA or non-U.S. regulatory authorities may disagree with the sufficiency of our right of reference to the preclinical, manufacturing or clinical data generated by these investigator-sponsored trials, or our interpretation of preclinical, manufacturing or clinical data from these investigator-sponsored trials. If so, the FDA or other non-U.S. regulatory authorities may require us to obtain and submit additional preclinical, manufacturing, or clinical data before we may initiate our planned trials and/or may not accept such additional data as adequate to initiate our planned trials.

We contract with third parties for the manufacture of our product candidates, including COPIKTRA, and for compound formulation research, and these third parties may not perform satisfactorily.

We do not have any manufacturing facilities or personnel. We currently obtain all of our supply of COPIKTRA and our other product candidates for clinical development from third-party manufacturers or third-party collaborators,

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and we expect to continue to rely on third parties for the manufacture of clinical quantities of our product candidates and commercial quantities of COPIKTRA. In addition, we currently rely on third parties for the development of various formulations of COPIKTRA and our other product candidates. This reliance on third parties increases the risk that we will not have sufficient quantities of COPIKTRA or our product candidates or such quantities at an acceptable cost or quality, which could delay, prevent or impair our development or commercialization efforts.

Any of these third parties may terminate their engagement with us at any time. We do not currently have arrangements in place for redundant supply or a second source for bulk drug substance. Even if we are able to establish agreements with third-party manufacturers, reliance on third-party manufacturers entails additional risks, including:

- reliance on the third party for regulatory compliance and quality assurance;
- the possible breach of the manufacturing agreement by the third party, including the misappropriation of our proprietary information, trade secrets and know how;
- the possible termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us; and
- disruptions to the operations of our manufacturers or suppliers caused by conditions unrelated to our business or operations, including the bankruptcy of the manufacturer or supplier or a catastrophic event affecting our manufacturers or suppliers.

Third-party manufacturers may not be able to comply with current good manufacturing practices (cGMP) regulations or similar regulatory requirements outside the United States. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our products and harm our business and results of operations.

Any products that we may develop may compete with other product candidates and products for access to manufacturing facilities. There are a limited number of manufacturers that operate under cGMP regulations and that might be capable of manufacturing for us. Any interruption of the development or operation of the manufacturing facilities due to, among other reasons, events such as order delays for equipment or materials, equipment malfunction, quality control and quality assurance issues, regulatory delays and possible negative effects of such delays on supply chains and expected timelines for product availability, production yield issues, shortages of qualified personnel, discontinuation of a facility or business or failure or damage to a facility by natural disasters, could result in the cancellation of shipments, loss of product in the manufacturing process or a shortfall in available COPIKTRA, other product candidates or materials.

If our current contract manufacturers cannot perform as agreed or these parties cease to provide quality manufacturing and related services to us, we may be required to replace that manufacturer. If we are not able to engage appropriate replacements in a timely manner, our ability to manufacture COPIKTRA or our other product candidates in sufficient quality and quantity required for commercial use of COPIKTRA and/or for planned pre-clinical testing, clinical trials and potential commercial use of our product candidates would be adversely affected. Although we believe that there are several potential alternative manufacturers who could manufacture our product candidates, we may incur added costs and delays in identifying and qualifying any such replacement, as well as producing the drug product and obtaining regulatory approvals for the new manufacturer. In addition, we have to enter into technical transfer agreements and share our know-how with the third-party manufacturers, which can be time consuming and may result in delays. In light of the lead time needed to manufacture COPIKTRA and our other product candidates, and the availability of underlying materials, we may not be able to, in a timely manner or at all, establish or maintain sufficient commercial manufacturing arrangements on commercially reasonable terms necessary to provide adequate supply of COPIKTRA to meet demands that exceed our commercial assumptions or to provide adequate supply of our other product candidates to meet demands that exceed our clinical assumptions. Furthermore, we may not be able to

obtain the significant financial capital that may be required in connection with such arrangements. Even after successfully engaging third parties to execute the manufacturing process for COPIKTRA and our other product candidates, such parties may not comply with the terms and timelines they have agreed to for various reasons, some of which may be out of their or our control, which

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could impact our ability to execute our business plans on expected or required timelines in connection with the commercialization of COPIKTRA and the continued development of our other product candidates. We may also be required to enter into long-term manufacturing agreements that contain exclusivity provisions and/or substantial termination penalties, which could have a material adverse effect on our business prior to and after commercialization.

Our current and anticipated future dependence upon others for the manufacture of our other product candidates or products may adversely affect our future profit margins and our ability to commercialize any products that receive marketing approval on a timely and competitive basis.

If we are not able to establish collaborations, we may have to alter our development and commercialization plans.

Our drug development programs and the potential commercialization of our product candidates will require substantial additional cash to fund expenses. For some of our product candidates, we may decide to collaborate with pharmaceutical and biotechnology companies for the development and potential commercialization of those product candidates.

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge and industry and market conditions generally. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us for our product candidate. Collaborations are complex and time consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of certain product candidates, reduce or delay our development programs, delay potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our product candidates or bring them to market and generate product revenue.

We may depend on collaborations with third parties for the commercialization of COPIKTRA and the development and commercialization of our other product candidates. If those collaborations are not successful, we may not be able to capitalize on the market potential of COPIKTRA or any other product candidates.

We may seek third-party collaborators for the development and commercialization of our product candidates. For instance, we have entered into agreements for the development and commercialization of COPIKTRA in China, Hong Kong, Macau and Taiwan with CSPC Pharmaceutical Group Limited and in Japan with Yakult Honsha Co., Ltd. We anticipate that we may seek to enter into a collaboration for marketing and commercialization of our product candidates in certain territories worldwide at the appropriate time in the future. Our likely collaborators for any collaboration arrangements include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies. If we do enter into any such arrangements with any third

parties, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of our product candidates. Our ability to generate revenues from these arrangements will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements.



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Collaborations involving our product candidates would pose the following risks to us:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not pursue development and commercialization of our product candidates or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborator's strategic focus or available funding or external factors such as an acquisition that diverts resources or creates competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing; collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our products or product candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- a collaborator with marketing and distribution rights to one or more products may not commit sufficient resources to the marketing and distribution of such product or products;
- collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our proprietary information or expose us to potential litigation;
- disputes may arise between the collaborators and us that result in the delay or termination of the research, development or commercialization of our products or product candidates or that result in costly litigation or arbitration that diverts management attention and resources; and
- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates.

Collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all. If a future collaborator of ours were to be involved in a business combination, the continued pursuit and emphasis on our product development or commercialization program could be delayed, diminished or terminated.

If we are unable to maintain our agreements with third parties to distribute COPIKTRA to patients, our results of operations and business could be adversely affected.

We will rely on third parties to commercially distribute COPIKTRA to patients. We have contracted with a third-party logistics company to warehouse COPIKTRA and to process and ship customer orders, and with specialty pharmacies and specialty distributors to sell and distribute COPIKTRA. The specialty pharmacies sell COPIKTRA directly to patients and provide patient education and ongoing management. The specialty distributors sell COPIKTRA to community oncologists with in-office dispensaries, hospital-owned practices, local offices with onsite pharmacies, retail pharmacies, and other institutional customers. We have also contracted with a third-party patient services hub to help us with some or all of the following: reimbursement adjudication, patient financial support, patient assistance programs and ongoing compliance support. This distribution network will require significant coordination with our sales and marketing and finance organizations. In addition, failure to coordinate financial systems could negatively impact our ability to accurately report product revenue from COPIKTRA. If we are unable to effectively manage the distribution process, the commercial launch and sales of COPIKTRA, as well as any future products we may commercialize, sales could be delayed or severely compromised and our results of operations may be harmed.

In addition, the use of specialty pharmacies, specialty distributors and a call center involves certain risks, including, but not limited to, risks that these organizations will:

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not provide us with accurate or timely information regarding their inventories, the number of patients who are using COPIKTRA or serious adverse reactions, events and/or product complaints regarding COPIKTRA;

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- not effectively sell or support COPIKTRA, or communicate publicly concerning COPIKTRA in a manner that is contrary to FDA rules and regulations;
- reduce or discontinue their efforts to sell or support COPIKTRA;
- not devote the resources necessary to sell COPIKTRA in the volumes and within the time frame we expect;
- be unable to satisfy financial obligations to us or others; or
- cease operations.

Any such events may result in decreased product sales and lower product revenue, which would harm our results of operations and business.

## Risks Related to Our Intellectual Property

If we fail to comply with our obligations under our intellectual property licenses with third parties, we could lose license rights that are important to our business.

We are a party to a number of intellectual property license agreements with third parties, including Infinity and Pfizer Inc., or Pfizer, and expect to enter into additional license agreements in the future. Our existing license agreements impose, and we expect that future license agreements will impose, various diligence, milestone payment, royalty, insurance and other obligations on us. For example, under our license agreements with Infinity and Pfizer, we are required to use diligent or commercially reasonable efforts to develop and commercialize licensed products under the agreement and to satisfy other specified obligations. If we fail to comply with our obligations under these licenses, our licensors may have the right to terminate these license agreements, in which event we might not be able to market any product that is covered by these agreements, or to convert the exclusive licenses to non-exclusive licenses, which could materially adversely affect the value of COPIKTRA or the product candidate being developed under these license agreements. Termination of these license agreements or reduction or elimination of our licensed rights may result in our having to negotiate new or reinstated licenses with less favorable terms, which may not be possible. If Pfizer were to terminate its license agreement with us for any reason, we would lose our rights to defactinib. If Infinity were to terminate its license agreement with us for any reason, we would lose our rights to COPIKTRA.

If we are unable to obtain and maintain patent protection for our products, or if our licensors are unable to obtain and maintain patent protection for the products that we license from them, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize products similar or identical to ours, and our ability to successfully commercialize our products may be adversely affected.

Our success depends in large part on our and our licensors' ability to obtain and maintain patent protection in the United States and other countries with respect to our products. We and our licensors seek to protect our proprietary position by filing patent applications in the United States and abroad related to our products that are important to our business. We cannot be certain that any patents will issue with claims that cover COPIKTRA or our other product candidates.

The patent prosecution process is expensive and time consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Moreover, in some circumstances, we do not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering products that we license from third parties and are reliant on our licensors. Therefore, we cannot be certain that these patents and applications will be prosecuted and enforced in a manner consistent with the best interests of our business. If such licensors fail to maintain such patents, or lose rights to those patents, the rights we have licensed may be reduced or eliminated.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our and our licensors' patent rights are highly uncertain. Our and

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our licensors' pending and future patent applications may not result in patents being issued which protect our products or which effectively prevent others from commercializing competitive products. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection.

The laws of foreign countries may not protect our rights to the same extent as the laws of the United States. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot be certain that we or our licensors were the first to make the inventions claimed in our owned or licensed patents or pending patent applications, or that we or our licensors were the first to file for patent protection of such inventions.

Assuming the other requirements for patentability are met, in the United States, for patents that have an effective filing date prior to March 15, 2013, the first to make the claimed invention is entitled to the patent, while outside the United States, the first to file a patent application is entitled to the patent. In March 2013, the United States transitioned to a first inventor to file system in which, assuming the other requirements for patentability are met, the first inventor to file a patent application will be entitled to the patent. We may be subject to a third-party pre-issuance submission of prior art to the U.S. Patent and Trademark Office, or become involved in opposition, derivation, reexamination, inter parties review or interference proceedings challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights.

Even if our owned and licensed patent applications issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our owned or licensed patents by developing similar or alternative technologies or products in a non-infringing manner.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, which could limit our ability to stop others from using or commercializing similar or identical products, or limit the duration of the patent protection of our products. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

We may become involved in lawsuits to protect or enforce our patents, which could be expensive, time consuming and unsuccessful.

Competitors may infringe our patents. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time consuming. In addition, in an infringement proceeding, a court may decide that a patent of ours is invalid or unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation proceeding could put one or more of our patents at risk of being invalidated or interpreted narrowly. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. In addition, our licensors may have rights to file and prosecute such claims and we are reliant on

them.

Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could have a material adverse effect on the success of our business.

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Our commercial success depends upon our ability and the ability of our collaborators to commercialize, develop, manufacture, market and sell COPIKTRA and our other product candidates without infringing the proprietary rights of third parties. We have yet to conduct comprehensive freedom to operate searches to determine whether our use of certain of the patent rights owned by or licensed to us would infringe patents issued to third parties. We may become party to, or threatened with, future adversarial proceedings or litigation regarding intellectual property rights with respect to our products, including interference proceedings before the U.S. Patent and Trademark Office. Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future. If we are found to infringe a third party's intellectual property rights, we could be required to obtain a license from such third party to continue developing and marketing our products. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. We could be forced, including by court order, to cease commercializing the infringing product. In addition, we could be found liable for monetary damages. A finding of infringement could prevent us from commercializing COPIKTRA and our other product candidates or force us to cease some of our business operations, which could materially harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business.

We may be subject to claims that our employees have wrongfully used or disclosed alleged trade secrets of their former employers.

Many of our employees were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees do not use the proprietary information or know how of others in their work for us, we may be subject to claims that we or these employees have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such employee's former employer. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

Intellectual property litigation could cause us to spend substantial resources and distract our personnel from their normal responsibilities.

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses, and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development, sales, marketing or distribution activities. We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have a material adverse effect on our ability to compete in the marketplace.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition to seeking patents for some of our products, we also rely on trade secrets, including unpatented know how, technology and other proprietary information, to maintain our competitive position. We seek to protect these trade

secrets, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, corporate collaborators, outside scientific collaborators, contract manufacturers, consultants, advisors and other third parties. We also enter into confidentiality and invention or patent assignment agreements with our employees and consultants. Despite these efforts, any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is



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difficult, expensive and time consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor, our competitive position would be harmed.

**Risks Related to Maintaining and Expanding COPIKTRA'S Regulatory Approval, Achieving Regulatory Approval of Our Other Product Candidates and Other Legal Compliance Matters**

If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals for our product candidates, we will not be able to commercialize such candidates, and our ability to generate revenue will be materially impaired.

Obtaining approval of an NDA can be a lengthy, expensive and uncertain process. The activities associated with a product candidate's development and commercialization, including its design, testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution are subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and by comparable authorities in other countries. Failure to obtain marketing approval for product candidates will prevent us from commercializing such product candidates. We have not received approval to market any of our product candidates from regulatory authorities in any jurisdiction, except for COPIKTRA in the United States. We have only limited experience in filing and supporting the applications necessary to gain marketing approvals and expect to rely on third-party contract research organizations to assist us in this process. Securing FDA approval requires the submission of extensive preclinical and clinical data and supporting information to the FDA for each therapeutic indication to establish the product candidate's safety and efficacy. Securing FDA approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the FDA. A product candidate may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our obtaining marketing approval or prevent or limit commercial use.

The process of obtaining marketing approvals, both in the United States and abroad, is expensive, may take many years if additional clinical trials are required, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Changes in marketing approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. The FDA has substantial discretion in the approval process and may refuse to accept any application or may decide that our data is insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent marketing approval of a product candidate. Any marketing approval we ultimately obtain may be subject to more limited indications than those we propose or subject to restrictions or post approval commitments that render the approved product not commercially viable.

If we experience delays in obtaining approval or if we fail to obtain approval of a product candidate, its commercial prospects may be harmed and our ability to generate revenues will be materially impaired.

We have received orphan drug designation for COPIKTRA and certain of our product candidates, but there can be no assurance that we will be able to prevent third parties from developing and commercializing products that are competitive to COPIKTRA or these product candidates.

We received orphan drug designation in the United States and the European Union for the use of COPIKTRA in CLL/SLL and FL, in the United States and European Union for the use of defactinib in ovarian cancer, and in the United States, the European Union, and Australia for the use of defactinib in mesothelioma. Orphan drug exclusivity grants seven years of marketing exclusivity under the Federal Food, Drug and Cosmetic Act (FDCA), up to ten years of marketing exclusivity in Europe, and five years of marketing exclusivity in Australia. Other companies have received orphan drug designations for compounds other than COPIKTRA or defactinib for the same indications for which we may

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have received orphan drug designation in corresponding territories. While orphan drug exclusivity for COPIKTRA or defactinib provides market exclusivity against the same active ingredient for the same indication, we would not be able to exclude other companies from manufacturing and/or selling drugs using the same active ingredient for the same indication beyond that timeframe on the basis of orphan drug exclusivity. Furthermore, the marketing exclusivity in Europe can be reduced from ten years to six years if the orphan designation criteria are no longer met or if the drug is sufficiently profitable so that market exclusivity is no longer justified. Even if we are the first to obtain marketing authorization for an orphan drug indication, there are circumstances under which the FDA may approve a competing product for the same indication during the seven-year period of marketing exclusivity, such as if the later product is the same compound as our product but is shown to be clinically superior to our product, or if the later product is a different drug than our product candidate. Further, the seven-year marketing exclusivity would not prevent competitors from obtaining approval of the same compound for other indications or of another compound for the same use as the orphan drug.

We may seek fast track designation for COPIKTRA in additional indications, or for one or more of our other product candidates, but we might not receive such designation, and even if we do, such designation may not actually lead to a faster development or regulatory review or approval process, and it does not ensure that we will receive marketing approval.

The FDA has granted fast track designation for COPIKTRA for the treatment of patients with peripheral T-cell lymphoma who have received at least one prior therapy. Any sponsor may seek fast track designation for a drug if it is intended for the treatment of a serious condition and nonclinical or clinical data demonstrate the potential to address unmet medical need for this condition, a drug sponsor may apply for FDA fast track designation. If we seek fast track designation for a product candidate, we may not receive it from the FDA. However, even if we receive fast track designation, fast track designation does not ensure that we will receive marketing approval or that approval will be granted within any particular timeframe. We may not experience a faster development or regulatory review or approval process with fast track designation compared to conventional FDA procedures. In addition, the FDA may withdraw fast track designation if it believes that the designation is no longer supported by data from our clinical development program. Fast track designation alone does not guarantee qualification for the FDA's priority review procedures.

COPIKTRA and any other product candidate for which we obtain marketing approval could be subject to restrictions or withdrawal from the market and we may be subject to penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our products.

COPIKTRA and any other product candidate for which we obtain marketing approval, along with the manufacturing processes, post approval clinical data, labeling, advertising and promotional activities for such product, will be subject to continual requirements of and review by the FDA and other regulatory authorities. These requirements include submissions of safety and other post marketing information and reports, registration and listing requirements, cGMP requirements relating to quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping. Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for costly post marketing testing and surveillance to monitor the safety or efficacy of the product, including the imposition of a REMS.

With respect to COPIKTRA, the indication in FL is approved by the FDA under accelerated approval based on overall response rate observed in clinical trials. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials. The FDA is requiring that we conduct a clinical trial in patients with relapsed or refractory FL that verifies and isolates the benefits of COPIKTRA. Additionally, as a requirement of the FDA's approval, we are implementing an informational REMS that entails a communication plan to

provide appropriate dosing and safety information to better support physicians in managing their patients on COPIKTRA.

The FDA closely regulates the post approval marketing and promotion of drugs to ensure drugs are marketed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA imposes

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stringent restrictions on manufacturers' communications regarding off label use and if we do not market our products for their approved indications, we may be subject to enforcement action for off label marketing.

In addition, later discovery of previously unknown problems with our products, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

- restrictions on such products, manufacturers or manufacturing processes;
- restrictions on the labeling or marketing of a product;
- restrictions on product distribution or use;
- requirements to conduct post marketing clinical trials;
- warning or untitled letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- fines, restitution or disgorgement of profits or revenue;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of our products;
- product seizure; or
- injunctions or the imposition of civil or criminal penalties.

The FDA's and other regulatory authorities' policies may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may fail to obtain any marketing approvals, lose any marketing approval that we may have obtained and we may not achieve or sustain profitability.

Our relationships with customers and third-party payors will be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and earnings.

Healthcare providers, including physicians, and third-party payors play a primary role in the recommendation and prescription of COPIKTRA and any other product candidates for which we obtain marketing approval. Our arrangements with healthcare providers, third-party payors and other parties within the healthcare industry may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute COPIKTRA and any products for which we obtain marketing approval. Restrictions under applicable federal and state healthcare and regulatory laws and regulations include the following:

- the federal healthcare anti-kickback statute prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under federal and state healthcare programs such as Medicare and Medicaid. A person or entity does not need to have actual knowledge of the anti-kickback statute or specific intent to violate it in order to have committed a violation;
- the federal False Claims Act (FCA), which imposes criminal and civil penalties on individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government and actions under the FCA may be brought by private whistleblowers as well as the government. In addition, the government may assert that a claim including items and services resulting from a violation of the federal anti-kickback statute constitutes a false or fraudulent claim for purposes of the FCA;



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- the federal civil monetary penalties laws, which impose civil fines for, among other things, the offering or transfer of remuneration to a Medicare or state healthcare program beneficiary if the person knows or should know it is likely to influence the beneficiary's selection of a particular provider, practitioner, or supplier of services reimbursable by Medicare or a state healthcare program, unless an exception applies;
- the federal Health Insurance Portability and Accountability Act of 1996 (HIPAA) imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program and HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, also establishes requirements related to the privacy, security and transmission of individually identifiable health information which apply to many healthcare providers, physicians and third-party payors with whom we interact;
- the federal false statements statute prohibits knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services;
- the FDCA, which among other things, strictly regulates drug product and medical device marketing, prohibits manufacturers from marketing such products for off-label use and regulates the distribution of samples;
- federal laws that require pharmaceutical manufacturers to report certain calculated product prices to the government or provide certain discounts or rebates to government authorities or private entities, often as a condition of reimbursement under governmental healthcare programs;
- federal and state consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers;
- the so-called federal "sunshine law" or Open Payments requires manufacturers of drugs, devices, biologics and medical supplies to report to the Department of Health and Human Services information related to payments and other transfers of value to physicians and teaching hospitals, as well as physician ownership and investment interests; and analogous state laws and regulations, such as state anti-kickback and false claims laws, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non- governmental third-party payors, including private insurers, and some state laws regulate interactions between pharmaceutical companies and healthcare providers and require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring drug manufacturers to report information related to payments to physicians and other healthcare providers or marketing expenditures and pricing information. State laws also govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Similar healthcare laws and regulations exist in the European Union and other foreign jurisdictions, including reporting requirements detailing interactions with and payments to healthcare providers and laws governing the privacy and security of certain protected information, such as GDPR, which imposes obligations and restrictions on the collection and use of personal data relating to individuals located in the EU (including health data).

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices, including arrangements we may have with physicians and other healthcare providers, or patient assistance programs, may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, exclusion from government funded healthcare programs, such as Medicare and Medicaid, additional reporting obligations and oversight if we become subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with these laws, and the curtailment or restructuring of our operations. If any of the physicians or other providers or entities with whom we expect to do business is found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs. Further, defending against any such actions can be costly, time-consuming and may require significant personnel resources. Therefore, even if we are successful in defending

against any such actions that may be brought against us, our business may be impaired.



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Our employees, independent contractors, principal investigators, CROs, consultants and vendors may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements, which could cause significant liability for us and harm our reputation.

We are exposed to the risk that our employees, independent contractors, principal investigators, CROs, consultants and vendors may engage in fraud or other misconduct, including intentional failures to: comply with FDA regulations or similar regulations of comparable foreign regulatory authorities, provide accurate information to the FDA or comparable foreign regulatory authorities, comply with manufacturing standards we have established, comply with federal and state healthcare fraud and abuse laws and regulations and similar laws and regulations established and enforced by comparable foreign regulatory authorities, report financial information or data accurately or disclose unauthorized activities to us. Such misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. It is not always possible to identify and deter misconduct by employees and other third parties, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws, standards or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business and results of operations, including the imposition of significant fines or other sanctions.

Recently enacted and future legislation may increase the difficulty and cost for us to commercialize COPIKTRA, obtain marketing approval of and commercialize our other product candidates and affect the prices we may obtain.

In the United States and some foreign jurisdictions, there have been, and we expect there will continue to be, a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could, among other things, prevent or delay marketing approval of our product candidates, restrict or regulate post approval activities and affect our ability to profitably sell COPIKTRA and any other product candidates for which we obtain marketing approval.

The U.S. healthcare industry generally and U.S. government healthcare programs in particular are highly regulated and subject to frequent and substantial changes. The U.S. government and individual states have been aggressively pursuing healthcare reform. For example, in March 2010, President Obama signed into law the Health Care Reform Act, a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. The law, for example, increased drug rebates under state Medicaid programs for brand name prescription drugs and extended those rebates to Medicaid managed care and assessed a fee on manufacturers and importers of brand name prescription drugs reimbursed under certain government programs, including Medicare and Medicaid.

Since its enactment, there have been ongoing judicial, legislative and administrative efforts to modify, repeal or prevent implementation of various provisions of the Health Care Reform Act. We cannot predict the ultimate content, timing or effect of any federal or state healthcare reform legislation or the impact of potential legislation on us.

In addition, other legislative changes have been proposed and adopted in the U.S. since the Health Care Reform Act was enacted. These changes included aggregate reductions to Medicare payments to providers of 2% per fiscal year, which went into effect in April 2013 and, due to the Bipartisan Budget Act of 2015, will remain in effect through 2027 unless additional action is taken by Congress. Tax reform legislation enacted at the end of 2017 eliminates the tax penalty for individuals who do not maintain sufficient health insurance coverage beginning in 2019 (the so-called "individual mandate").

Individual states in the United States have also become increasingly active in passing legislation and implementing regulations designed to control pharmaceutical product pricing, including price constraints, restrictions on copayment assistance by pharmaceutical manufacturers, marketing cost disclosure and transparency measures, and, in some cases, measures designed to encourage importation from other countries and bulk purchasing. In addition,

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individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs.

We cannot be sure whether additional legislative changes will be enacted, or whether the regulations, guidance or interpretations will be changed, or what the impact of such changes on COPIKTRA or the marketing approvals of our product candidates may be. In addition, increased scrutiny by the U.S. Congress of the FDA's approval process may significantly delay or prevent marketing approval, as well as subject us to more stringent product labeling and post marketing testing and other requirements.

### Risks Related to Employee Matters and Managing Growth

Our future success depends on our ability to retain our chief executive officer and other key executives and to attract, retain and motivate qualified personnel.

We are highly dependent on Robert Forrester, our President and Chief Executive Officer, Daniel Paterson, our Chief Operating Officer, Robert Gagnon, our Chief Financial Officer, and Joseph Lobacki, our Chief Commercial Officer, as well as the other principal members of our management and scientific teams. Although we have formal employment agreements with Robert Forrester, Daniel Paterson, Robert Gagnon and Joseph Lobacki, these agreements do not prevent them from terminating their employment with us at any time. We do not maintain "key person" insurance for any of our executives or other employees. The loss of the services of any of these persons could impede the achievement of our research, development and commercialization objectives.

Recruiting and retaining qualified scientific, clinical, manufacturing and sales and marketing personnel will also be critical to our success. We may not be able to attract and retain these personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies, universities and research institutions for similar personnel. Although we have implemented a retention plan for certain key employees, our retention plan may not be successful in incentivizing these employees to continue their employment with us. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors, including our scientific co-founders, may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us.

We may expand our development, regulatory and sales and marketing capabilities over time, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

We may experience significant growth over time in the number of our employees and the scope of our operations, particularly in the areas of drug development, regulatory affairs and sales and marketing. To manage our anticipated future growth, we may continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel when we expand. The physical expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

Our business and operations may be materially adversely affected in the event of computer system breaches or failures.

Despite the implementation of security measures, our internal computer systems, and those of our contract research organizations and other third parties on which we rely, are vulnerable to damage from computer viruses, unauthorized access, natural disasters, fire, terrorism, war and telecommunication and electrical failures. If such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our key business

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processes and clinical development programs. For example, the loss of clinical trial data from ongoing or planned clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach results in a loss of or damage to our data or applications, or inappropriate disclosure of confidential or proprietary information, we could be exposed to liability, which could have a material adverse effect on our operating results and financial condition and possibly delay the further development and commercialization of COPIKTRA and our other product candidates.

### Risks Related to Our Common Stock

Provisions in our corporate charter documents and under Delaware law could make an acquisition of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.

Provisions in our corporate charter and our bylaws may discourage, delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which you might otherwise receive a premium for your shares. These provisions could also limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our board of directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions:

- establish a classified board of directors such that not all members of the board are elected at one time;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from the board;
- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a "poison pill" that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 75% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our charter or bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner.

The market price of our common stock has been, and may continue to be, highly volatile.

Our stock price has been volatile. Since January 27, 2012, when we became a public company, the price for one share of our common stock has reached a high of \$18.82 and a low of \$1.05 through September 30, 2018. We cannot predict whether the price of our common stock will rise or fall. The market price for our common stock may be influenced by many factors, including:

- the success of competitive products or technologies;
-

results of clinical trials of our product candidates or those of our competitors; the success of commercializing COPIKTRA;

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- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our product candidates or clinical development programs;
- the results of our efforts to discover, develop, acquire or in-license additional product candidates or products;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions; and
- the other factors described in this "Risk Factors" section.

In addition, the stock market in general and the market for small pharmaceutical companies and biotechnology companies in particular have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of particular companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance. In the past, following periods of volatility in the market, securities class action litigation has often been instituted against companies. Such litigation, if instituted against us, could result in substantial costs and diversion of management's attention and resources, which could materially and adversely affect our business and financial condition.

Because we do not anticipate paying any cash dividends on our capital stock in the foreseeable future, capital appreciation, if any, will be the source of gain for our stockholders.

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all of our future earnings to finance the growth and development of our business. In addition, the terms of any current or future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be the sole source of gain for our stockholders for the foreseeable future.

We have limited experience in marketing and commercializing product candidates. If we are unable to successfully maintain and further develop internal commercialization capabilities, establish sales and marketing capabilities or enter into agreements with third parties to sell and market our product candidates, sales of COPIKTRA may be negatively impacted and we may not be successful in commercializing our other product candidates if and when they are approved.

We have limited experience in the sale, marketing or distribution of pharmaceutical products. To achieve commercial success for any approved product, we must either develop a sales and marketing organization or outsource these functions to third parties.

We have hired a commercial team and implemented the organizational infrastructure we believe we need for a successful commercial launch of COPIKTRA. We will need to commit significant time and financial and managerial resources to maintain and further develop our marketing and sales force to ensure they have the technical expertise required to address any challenges we may face with the commercialization of COPIKTRA. Factors that may inhibit our efforts to maintain and develop our commercialization capabilities include:

- an inability to retain an adequate number of effective commercial personnel;
- an inability to train sales personnel, who may have limited experience with our company or COPIKTRA, to deliver a consistent message regarding COPIKTRA and be effective in persuading physicians to prescribe COPIKTRA;
- an inability of sales personnel to obtain access to physicians or persuade adequate numbers of physicians to prescribe COPIKTRA or any other product candidates;





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- an inability of third-parties to manufacture COPIKTRA consistently in commercial quantities, at acceptable costs and on expected timelines;
- a lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines;
- an inability to equip sales personnel with effective materials, including medical and sales literature to help them educate physicians and other healthcare providers regarding COPIKTRA; and
- unforeseen costs and expenses associated with maintaining and further developing an independent sales and marketing organization.

If we are not successful in establishing and maintaining an effective sales and marketing infrastructure, we will have difficulty commercializing COPIKTRA, which would adversely affect our business and financial condition.

If we enter into arrangements with third parties to perform sales, marketing and distribution services, our product revenues or the profitability of these product revenues to us are likely to be lower than if we were to market and sell any products that we develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell and market our product candidates or may be unable to do so on terms that are favorable to us. We likely will have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively. If we do not establish sales and marketing capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our product candidates.

## Risks Related to the Notes

Servicing our debt, including the Notes, requires a significant amount of cash, and we may not have sufficient cash flow from our business to pay our substantial debt.

Our ability to make scheduled payments of the principal of, to pay interest on or to refinance our indebtedness, including the Notes, depends on the timing of regulatory reviews and approvals and our future performance, which is subject to regulatory, economic, financial, competitive and other factors beyond our control. We are a clinical stage biopharmaceutical company and we have not yet generated any profit from product sales. We expect to continue to incur losses as we continue our clinical development of, and seek regulatory approvals for, our product candidates, prepare to commercialize any approved products and add infrastructure and personnel to support our product development efforts and operations. Accordingly, our business may not generate cash flow from operations in the future sufficient to service our debt and make necessary capital expenditures. If we are unable to generate such cash flow, we may be required to adopt one or more alternatives, such as selling assets, restructuring debt or obtaining additional equity capital on terms that may be onerous or highly dilutive. Our ability to refinance our indebtedness will depend on the capital markets and our financial condition at such time. We may not be able to engage in any of these activities or engage in these activities on desirable terms, which could result in a default on our debt obligations.

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The Notes are effectively subordinated to our secured indebtedness and structurally subordinated to any liabilities of our subsidiaries.

The Notes are our senior, unsecured obligations and are senior in right of payment to our future indebtedness that is expressly subordinated in right of payment to the Notes; equal in right of payment with our existing and future indebtedness that is not so subordinated, and effectively subordinated to our existing and future secured indebtedness, to the extent of the value of the collateral securing such indebtedness. The Notes are structurally subordinated to all existing and future indebtedness and other liabilities, including trade payables, and (to the extent we are not a holder thereof) preferred equity, if any, of our subsidiaries. In the event of our bankruptcy, liquidation, reorganization or other winding up, our assets that secure debt will be available to pay obligations on the Notes only after the secured debt has been repaid in full from these assets, and the assets of our subsidiaries will be available to pay obligations on the Notes only after all claims of such subsidiaries' creditors, including trade creditors and preferred equity holders have been repaid in full. There may not be sufficient assets remaining to pay amounts due on any or all of the Notes then outstanding. The indenture and supplemental indenture governing the Notes do not prohibit us from incurring additional senior debt or secured debt, nor do they prohibit any of our subsidiaries from incurring additional liabilities.

Despite our current debt levels, we may still incur substantially more debt or take other actions which would intensify the risks discussed above.

Despite our current consolidated debt levels, we and our subsidiaries may be able to incur substantial additional debt in the future, subject to the restrictions contained in our debt agreements, some of which may be secured debt. We are not restricted under the terms of the indenture or the supplemental indenture governing the Notes from incurring additional debt, securing existing or future debt, recapitalizing our debt or taking a number of other actions that are not limited by the terms of the indenture or the supplemental indenture governing the Notes that could have the effect of diminishing our ability to make payments on the Notes when due. While the Amended Loan Agreement, as amended by the Third Amendment, restricts our ability and the ability of our subsidiaries to issue or incur additional indebtedness, including secured indebtedness, if our loans under the Amended Loan Agreement, as amended by the Third Amendment, mature or are repaid, we may not be subject to such restrictions under the terms of any subsequent indebtedness.

We may not have the ability to raise the funds necessary to repurchase the Notes upon a fundamental change, and our existing or future debt may contain limitations on our ability to repurchase the Notes.

Holders of the Notes have the right to require us to repurchase their Notes upon the occurrence of a fundamental change at a fundamental change repurchase price equal to 100% of the principal amount of the Notes to be repurchased, plus accrued and unpaid interest, if any. However, we may not have enough available cash or be able to obtain financing at the time we are required to make repurchases of Notes surrendered therefor. In addition, our ability to repurchase the Notes may be limited by law, by regulatory authority or by agreements governing our indebtedness that exist at the time of the repurchase. The Amended Loan Agreement, as amended by the Third Amendment, currently limits our ability to repurchase the Notes. Our failure to repurchase Notes at a time when the repurchase is required by the indenture and supplemental indenture governing the Notes would constitute a default under the indenture and supplemental indenture. A default under the indenture or the supplemental indenture or the fundamental change itself could also lead to a default under the Amended Loan Agreement, as amended by the Third Amendment, and/or agreements governing our future indebtedness. If the repayment of the related indebtedness were to be accelerated after any applicable notice or grace periods, we may not have sufficient funds to repay the indebtedness and repurchase the Notes.

In addition, our borrowings under the Amended Loan Agreement, as amended by the Third Amendment, are, and are expected to continue to be, at variable rates of interest and expose us to interest rate risk. If interest rates increase, our

debt service obligations on the variable rate indebtedness would increase even though the amount borrowed remained the same, and our net income would decrease.

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The Amended Loan Agreement, as amended by the Third Amendment, limits our ability to pay any cash amount upon repurchase of the Notes.

The Amended Loan Agreement, as amended by the Third Amendment, prohibits us from making any cash payments to repurchase the Notes upon a fundamental change. Any new credit facility that we may enter into may have similar restrictions.

Our failure to repurchase the Notes as required under the terms of the Notes would constitute a default under the indenture and the supplemental indenture governing the Notes and would permit holders of the Notes to accelerate our obligations under the Notes. A default under the indenture or the supplemental indenture or the fundamental change itself could also lead to a default under the Amended Loan Agreement, as amended by the Third Amendment, or agreements governing our future indebtedness. If the repayment of the related indebtedness were to be accelerated after any applicable notice or grace periods, we may not have sufficient funds to repay the indebtedness and repurchase the Notes.

Future sales of our common stock or equity-linked securities in the public market could lower the market price for our common stock.

In the future, we may sell additional shares of our common stock or equity-linked securities to raise capital. In addition, a substantial number of shares of our common stock are reserved for issuance upon the exercise of stock options and upon conversion of the Notes. We cannot predict the size of future issuances or the effect, if any, that they may have on the market price for our common stock. The issuance and sale of substantial amounts of common stock or equity-linked securities, or the perception that such issuances and sales may occur, could adversely affect the market price of our common stock and impair our ability to raise capital through the sale of additional equity or equity-linked securities.

## 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.

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Item 5. Other Information.

The following disclosure is provided in accordance with and in satisfaction of the requirements of Item 2.02 “Results of Operations and Financial Condition” of Form 8-K:

On November 7, 2018, Verastem, Inc. announced its financial results for the quarter ended September 30, 2018 and commented on certain corporate accomplishments and plans. The full text of the press release issued in connection with the announcement is furnished as Exhibit 99.1 hereto.

The information furnished in Item 5 (including Exhibit 99.1) shall not be deemed “filed” for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, except as expressly set forth by specific reference in such a filing.

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.

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EXHIBIT INDEX

4.1	<u>Indenture, dated as of October 17, 2018, by and between the Company and Wilmington Trust, National Association (incorporated by reference to Exhibit 4.1 to Form 8-K filed by the Registrant on October 17, 2018).</u>
4.2	<u>First Supplemental Indenture, dated as of October 17, 2018, by and between the Company and Wilmington Trust, National Association (incorporated by reference to Exhibit 4.2 to Form 8-K filed by the Registrant on October 17, 2018).</u>
4.3	* <u>Form of Inducement Award Restricted Stock Unit Agreement.</u>
10.1	*† <u>License and Collaboration Agreement, dated September 25, 2018, between Verastem, Inc. and CSPC Pharmaceutical Group Limited.</u>
10.2	* <u>Consulting Agreement, dated October 3, 2018, between Verastem, Inc. and Louise Phanstiel.</u>
10.3	<u>Amendment No. 3 to Loan and Security Agreement, as amended, with Hercules Capital, Inc., as administrative agent, and the Lenders from time to time party thereto (incorporated by reference to Exhibit 10.1 to Form 8-K filed by the Registrant on October 11, 2018).</u>
31.1	* <u>Certification of Chief 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.</u>
31.2	* <u>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.</u>
32.1	* <u>Certification of Chief Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.</u>
32.2	* <u>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.</u>
99.1	* <u>Press Release issued by Verastem, Inc. on November 7, 2018.</u>
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.

†Confidential treatment requested under 17 C.F.R. §200.80(c) and Rule 24b-2. The confidential portions of this exhibit have been omitted and are marked accordingly. The confidential portions have been provided separately to the SEC pursuant to the confidential treatment request.

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SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

VERASTEM, INC.

Date: November 7, 2018 By: /s/ ROBERT FORRESTER

Robert Forrester  
President and Chief Executive Officer  
(Principal executive officer)

Date: November 7, 2018 By: /s/ ROBERT GAGNON

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