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.

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	$ —

Supplemental disclosure of non-cash financing activities		
Common stock issuance costs included in accounts payable and accrued expenses	$ 15	$ —

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):

	September 30, 2018			
Description	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	$ —

	December 31, 2017			
Description	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	$ —

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

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)	

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;
-

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