Karyopharm Therapeutics Inc.
Form 10-Q
May 10, 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 March 31, 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: 001-36167

Karyopharm Therapeutics Inc.

(Exact name of registrant as specified in its charter)

Delaware	**26-3931704**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification Number)**
85 Wells Avenue, 2nd Floor	
Newton, MA	**02459**
(Address of principal executive offices)	**(Zip Code)**

(617) 658-0600

(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 and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post 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 (Do not check if a smaller reporting company)	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 May 4, 2018, there were 49,849,972 shares of Common Stock, $0.0001 par value per share, outstanding.

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PART I FINANCIAL INFORMATION

Item 1. Condensed Consolidated Financial Statements (Unaudited).

Karyopharm Therapeutics Inc.

CONDENSED CONSOLIDATED BALANCE SHEETS

(unaudited)

(in thousands, except share and per share amounts)

	March 31, 2018	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$ 37,499	$ 68,997
Short-term investments	93,418	77,472
Prepaid expenses and other current assets	2,396	1,754
Restricted cash		200
Total current assets	133,313	148,423
Property and equipment, net	2,454	2,185
Long-term investments	10,314	29,396
Restricted cash	292	290
Total assets	$ 146,373	$ 180,294
Liabilities and stockholders equity		
Current liabilities:		
Accounts payable	$ 4,949	$ 5,665
Accrued expenses	21,545	21,445
Deferred revenue	19,729	21,921
Deferred rent	178	303
Other current liabilities	333	133
Total current liabilities	46,734	49,467
Deferred revenue, net of current portion	2,192	
Deferred rent, net of current portion	1,918	1,363
Total liabilities	50,844	50,830
Stockholders equity:		
Preferred stock, $0.0001 par value; 5,000,000 shares authorized; none issued and outstanding		
	5	5

Common stock, $0.0001 par value; 100,000,000 shares authorized; 49,670,328 and 49,533,150 shares issued and outstanding at March 31, 2018 and December 31, 2017, respectively		
Additional paid-in capital	629,610	625,017
Accumulated other comprehensive loss	(286)	(217)
Accumulated deficit	(533,800)	(495,341)
Total stockholders equity	95,529	129,464
Total liabilities and stockholders equity	$ 146,373	$ 180,294

See accompanying notes to condensed consolidated financial statements.

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Karyopharm Therapeutics Inc.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS

(unaudited)

(in thousands, except share and per share amounts)

	Three Months Ended, March 31,	
	2018	**2017**
License and other revenue	$ 10,000	$ 68
Operating expenses:		
Research and development	41,321	24,083
General and administrative	7,621	6,264
Total operating expenses	48,942	30,347
Loss from operations	(38,942)	(30,279)
Other income (expense):		
Interest income	509	400
Other expense	(14)	(15)
Total other income, net	495	385
Loss before income taxes	(38,447)	(29,894)
Provision for income taxes	(12)	(23)
Net loss	$ (38,459)	$ (29,917)
Net loss per share basic and diluted	$ (0.78)	$ (0.71)
Weighted-average number of common shares outstanding used in net loss per share basic and diluted	49,602,809	41,894,796

See accompanying notes to condensed consolidated financial statements.

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Karyopharm Therapeutics Inc.

CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS

(unaudited)

(in thousands)

	Three Months Ended March 31,	
	2018	**2017**
Net loss	$ (38,459)	$ (29,917)
Comprehensive income (loss)		
Unrealized gain (loss) on investments	(108)	59
Foreign currency translation adjustments	39	11
Comprehensive loss	$ (38,528)	$ (29,847)

See accompanying notes to condensed consolidated financial statements.

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Karyopharm Therapeutics Inc.

CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS

(unaudited)

(in thousands)

	Three Months Ended March 31,	
	2018	**2017**
Operating activities		
Net loss	$ (38,459)	$ (29,917)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	169	183
Net amortization of premiums and discounts on investments	160	267
Stock-based compensation expense	4,164	5,909
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(638)	(61)
Accounts payable	(773)	(522)
Accrued expenses and other liabilities	295	(498)
Deferred rent	430	(69)
Net cash used in operating activities	(34,652)	(24,708)
Investing activities		
Purchases of property and equipment	(382)	
Proceeds from maturities of investments	27,602	25,624
Purchases of investments	(24,736)	(25,075)
Net cash provided by investing activities	2,484	549
Financing activities		
Proceeds from the exercise of stock options	429	57
Net cash provided by financing activities	429	57
Effect of exchange rate on cash	43	16
Net decrease in cash and cash equivalents	(31,696)	(24,086)
Cash, cash equivalents and restricted cash at beginning of period	69,487	50,142
Cash, cash equivalents and restricted cash at end of period	$ 37,791	$ 26,056
Supplemental disclosure of non-cash activities		
Purchases of property and equipment included in accounts payable	$ 56	$
Deferred financing costs included in accounts payable	$	$ 15

See accompanying notes to condensed consolidated financial statements.

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Karyopharm Therapeutics Inc.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

(in thousands except share and per share data)

1. Summary of Significant Accounting Policies

Basis of Presentation

The accompanying unaudited condensed consolidated financial statements of Karyopharm Therapeutics Inc., a Delaware corporation (the Company), have been prepared in accordance with accounting principles generally accepted 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 of assets, liabilities, revenues, expenses and related disclosures at the date of the financial statements. Actual results could differ from those estimates. Additionally, operating results for the three months ended March 31, 2018 are not necessarily indicative of the results that may be expected for any other interim period or for the fiscal 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 15, 2018.

Basis of Consolidation

The condensed consolidated financial statements at March 31, 2018 include the accounts of (i) the Company, (ii) Karyopharm Securities Corp. (a wholly-owned Massachusetts corporation of the Company incorporated in December 2013), (iii) Karyopharm Europe GmbH (a wholly-owned German Limited Liability Company formed in August 2014) and (iv) Karyopharm Therapeutics (Bermuda) Ltd. (a wholly-owned Bermuda subsidiary of the Company formed in March 2015). All intercompany balances and transactions have been eliminated in consolidation.

Revenue Recognition

The Company adopted ASU 2014-09, *Revenue from Contracts with Customers (*ASC 606), as well as subsequent amendments, which were codified in ASC 606, on January 1, 2018, using the modified retrospective method for all contracts not completed as of the date of adoption. The reported results for the quarter ended March 31, 2018 reflect the application of ASC 606 while the reported results for 2017 were prepared under the guidance of ASC 605, *Revenue Recognition* (ASC 605), which is also referred to herein as legacy GAAP or the previous guidance . The adoption of ASC 606 did not have a material impact on the Company s consolidated financial position, results of operations, stockholder s equity or cash flows as of the adoption date, as no transition adjustment for any of the Company s contracts with customers was required.

ASC 606 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. At contract inception, once the contract is determined to be within the scope of ASC 606, the Company assesses the goods or services promised within each contract and determines those that 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.

The Company generates revenue from license or similar agreements with pharmaceutical companies for the development and commercialization of certain of its product candidates. Such agreements may include the transfer of intellectual property rights in the form of licenses, transfer of technological know-how, delivery of drug substances, research and development services, and participation on certain committees with the counterparty. Payments made by the customers may include non-refundable upfront fees, payments upon the excercise of customer options, payments based upon the achievement of defined milestones, and royalties on sales of product candidates if they are successfully approved and commercialized.

If the license to the Company s intellectual property is determined to be distinct from the other performance obligations identified in the arrangement, the Company recognizes the transaction price allocated to the license as revenue upon transfer of control of the license. The Company evaluates all other promised goods or services in the agreement to determine if they are distinct. If they are not distinct, they are combined with other promised goods or services to create a bundle of promised goods or services that is

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distinct. Optional future services where any additional consideration paid to the Company reflects their standalone selling prices do not provide the customer with a material right and, therefore, are not considered performance obligations. If optional future services are priced in a manner which provides the customer with a significant or incremental discount, they are material rights, and are accounted for as performance obligations.

The Company utilizes judgment to determine the transaction price. In connection therewith, the Company evaluates contingent milestones at contract inception to estimate the amount which is not probable of a material reversal to include in the transaction price using the most likely amount method. Milestone payments that are not within the control of the Company, such as regulatory approvals, are not considered probable of being achieved until those approvals are received and therefore the variable consideration is constrained. The transaction price is then allocated to each performance obligation on a relative stand-alone selling price basis, for which the Company recognizes revenue as or when the performance obligations under the contract are satisfied. At the end of each reporting period, the Company re-evaluates the probability of achieving development milestone payments which may not be subject to a material reversal 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 license and other revenue, as well as earnings, in the period of adjustment.

The Company then determines whether the performance obligations or combined performance obligations are satisfied over time or at a point in time and, if over time, the appropriate method of measuring progress for purposes of recognizing revenue from non-refundable, upfront fees. The Company evaluates the measure of progress, as applicable, each reporting period and, if necessary, adjusts the measure of performance and related revenue recognition.

When consideration is received, or such consideration is unconditionally due, from a customer prior to transferring goods or services to the customer under the terms of a contract, a contract liability is recorded within deferred revenue. Contract liabilities within deferred revenue are recognized as revenue after control of the goods or services is transferred to the customer and all revenue recognition criteria have been met.

For arrangements that include sales-based royalties, including sales-based milestone payments, and the license is deemed to be the predominant item to which the royalties relate, the Company recognizes revenue at the later of when the related sales occur or when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied).

2. Recent Accounting Pronouncements

Recently Adopted Accounting Standards

As detailed above, the Company adopted ASC 606 on January 1, 2018. Under the modified retrospective transition method, the Company applied ASC 606 to all contracts within scope as of January 1, 2018. Under the practical expedient concerning contract modifications contained in the transitional provisions of ASC 606, the Company has not retrospectively restated its contracts for modifications prior to the earliest period presented, and instead has reflected the aggregate effect of all modifications when identifying the satisfied and unsatisfied performance obligations, determining the transaction price and allocating the transaction price. Qualitatively, the effect of applying this practical expedient is not material to the periods presented in the consolidated financial statements. As more fully discussed in Note 3, Asset Purchase and License Agreements, only the Company s arrangement with Ono Pharmaceutical Co., Ltd. was determined to have unsatisfied performance obligations as of the adoption date. However, the pattern of revenue recognition was not affected and, therefore, no transition adjustment was recorded to the opening balance of accumulated deficit on January 1, 2018. All other agreements subject to transition, which only

included the Company s arrangement with Anivive Lifesciences Inc., were unaffected by the adoption of ASC 606 in all periods presented in the consolidated financial statements through application of the modified retrospective transition method.

In August 2016, the FASB issued ASU 2016-15, *Classification of Certain Cash Receipts and Cash Payments* (ASU 2016-15). ASU 2016-15. This standard addresses eight specific cash flow issues with the objective of reducing the existing diversity in practice. The Company adopted ASU 2016-05 effective January 1, 2018 and the adoption did not have a material impact on the Company s statements of cash flows.

In October 2016, the FASB issued Accounting Standards Update (ASU) No. 2016-16, *Accounting for Income Taxes: Intra-Entity Asset Transfers of Assets Other than Inventory (Topic 740)*. Topic 740 eliminates the ability to defer the tax expense related to intra-entity asset transfers other than inventory. Under the new standard, entities should recognize the income tax consequences on an intra-entity transfer of an asset other than inventory when the transfer occurs. The Company adopted Topic 740 effective January 1, 2018 and the adoption did not have a material impact on the Company s financial position or results of operations.

In November 2016, the FASB issued ASU No. 2016-18, *Statement of Cash Flows (Topic 230): Restricted Cash*. The new standard 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. Therefore, 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-

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period total amounts shown on the statement of cash flows. The Company adopted this standard effective January 1, 2018 and reclassified restricted cash in the statements of cash flows to be included in the cash and cash equivalents balance. The standard resulted in the reclassification of $292 and $479 into the balance of cash, cash equivalents and restricted cash on the statement of cash flows for the periods ended March 31, 2018 and 2017, respectively.

In May 2017, the FASB issued ASU 2017-09, *Compensation-Stock Compensation (Topic 718)* (ASU 2017-09) *Scope of Modification Accounting*. ASU 2017-09 provides clarification on when modification accounting should be used for changes to the terms or conditions of a share-based payment award. This ASU does not change the accounting for modifications but clarifies that modification accounting guidance should only be applied if there is a change to the value, vesting conditions, or award classification and would not be required if the changes are considered non-substantive. The Company adopted this standard effective January 1, 2018 and the adoption did not have a material impact on the Company s consolidated financial statements.

Recently Issued Accounting Standards

In February 2016, the FASB issued ASU No. 2016-02, *Leases (Topic 842)*. The new standard requires that all lessees recognize the assets and liabilities that arise from leases on the balance sheet and disclose qualitative and quantitative information about its leasing arrangements. The new standard will be effective for the Company on January 1, 2019. The Company is in process of evaluating this guidance and determining the potential impact on its consolidated financial statements; however, it anticipates that the new standard will result in the Company recording additional right of use assets and corresponding liabilities on its consolidated balance sheet.

3. Asset Purchase and License Agreements

Biogen Asset Purchase Agreement

On January 24, 2018, the Company entered into an Asset Purchase Agreement (the APA) and Letter Agreement with Biogen MA Inc., a Massachusetts corporation and subsidiary of Biogen, Inc. (Biogen).

Under the terms of the APA and Letter Agreement, the Company sold Biogen exclusive worldwide rights to develop and commercialize the Company s oral Selective Inhibitor of Nuclear Export (SINE) compound KPT-350 and certain related assets with an initial focus in amyotrophic lateral sclerosis (ALS) (the Transfer of IP), and also granted Biogen: (i) an exclusive worldwide license under certain of the Company s intellectual property to manufacture or have manufactured KPT-350 (the Manufacturing License), (ii) a technology transfer package, consisting of information and the Company s know-how regarding the manufacture of KPT-350 (the Manufacturing Technology Transfer), (iii) a right, at Biogen s request, to have the Company provide transition assistance regarding manufacturing and other matters (the Transition Assistance), (iv) existing inventory of KPT-350 (the Inventory), (v) an initial supply of KPT-350 (the Initial Supply), and (vi) a right, at Biogen s request, to have the Company manufacture and supply the active pharmaceutical ingredient for an additional supply of KPT-350 (the Additional Supply). In consideration for these rights, the Company received an upfront payment of $10,000, and is eligible to receive additional payments of up to $142,000 based on the achievement by Biogen of future specified development milestones, and up to $65,000 based on the achievement by Biogen of future specified commercial milestones. The Company will also be eligible to receive tiered royalty payments that reach low double-digits based on future net sales until the later of the tenth anniversary of the first commercial sale of the applicable product and the expiration of specified patent protection for the applicable product, determined on a country-by-country basis.

The Company and Biogen have made customary representations and warranties and agreed to customary covenants in the APA, including covenants requiring Biogen to use commercially reasonable efforts to develop KPT-350 in

specified neurological indications, including ALS, in any of the United States, United Kingdom, France, Spain, Germany or Italy. The APA will continue in effect until the expiration of all royalty obligations, provided that the APA may be terminated earlier by Biogen, subject to the requirements that Biogen (i) negotiate in good faith with the Company regarding an assignment or license back to the Company of the purchased assets and (ii) not transfer or license the purchased assets to a third party unless such third party assumes Biogen s obligations to the Company under the APA.

The Company assessed this arrangement in accordance with ASC 606 and concluded that the contract counterparty, Biogen, is a customer. The Company identified the following material promises in the arrangement: the Transfer of IP and the Manufacturing License. The Company also identified other immaterial promises under the contract that were not deemed performance obligations. The Company further determined other promises for Additional Supply and Transition Assistance represented customer options, which would create an obligation for the Company if exercised by Biogen. Since either no additional or immaterial consideration is owed to the Company by Biogen upon exercise of the customer options for Additional Supply and Transition Assistance, the Company determined both are offered at significant and incremental discounts. Accordingly, they were assessed as material rights and, therefore, separate performance obligations in the arrangement.

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The Company then determined the Transfer of IP and the Manufacturing License were not distinct from one another and must be combined as a performance obligation (the Combined Performance Obligation). This is because Biogen requires the Manufacturing License to derive benefit from the Transfer of IP. Based on these determinations, as well as the considerations noted above with respect to the material rights for Additional Supply and Transition Assistance, the Company identified three distinct performance obligations at the inception of the contract: (i) the Combined Performance Obligation, (ii) the material right for Additional Supply, and (iii) the material right for Transition Assistance.

The Company further determined that the up-front payment of $10,000 constituted the entirety of the consideration to be included in the transaction price at contract inception, which was allocated to the performance obligations based on their relative stand-alone selling prices. In connection therewith, the Company estimated the stand-alone selling price of the (i) Combined Performance Obligation, (ii) material right for Additional Supply, and (iii) material right for Transition Assistance, and determined the stand-alone selling price of the material rights for Additional Supply and Transition Assistance were insignificant based on various quantitative and qualitative considerations. Accordingly, the Company further determined the allocation of the transaction price to the material rights for Additional Supply and Transition Assistance was insignificant. Based on the estimates of the stand-alone selling prices for each of the performance obligations, the Company determined that substantially all the $10,000 transaction price should be allocated to the Combined Performance Obligation. The Company believes that a change in the assumptions used to determine its best estimate of the stand-alone selling prices for the identified performance obligations would not have a significant effect on the allocation of the underlying transaction price to the performance obligations.

Upon execution of the Biogen Agreement, the transaction price included only the $10,000 up-front payment owed to the Company. The Company may receive further payments upon the achievement of certain regulatory and sales milestones, as detailed above, as well as tiered royalty payments that reach low double-digits based on future net sales. The future regulatory milestones, which represent variable consideration, were evaluated under the most likely amount method, and were not included in the transaction price, because the amounts are fully constrained as of March 31, 2018. As part of its evaluation of the constraint, the Company considered numerous factors, including that receipt of such milestones is outside the control of the Company. Separately, any consideration related to sales-based milestones, as well as royalties on net sales upon commercialization by Biogen, will be recognized when the related sales occur, as they were determined to relate predominantly to the intellectual property and, therefore, have also been excluded from the transaction price in accordance with the sales-based royalty exception, as well as the Company s accounting policy. The Company will re-evaluate the transaction price in each reporting period, as uncertain events are resolved, or as other changes in circumstances occur.

During the quarter ended March 31, 2018, the Company recognized $10,000 of revenue, as it had satisfied its promises under the Combined Performance Obligation by transferring the underlying promised goods at a point in time during the quarter ended March 31, 2018.

Ono License Agreement

Effective October 11, 2017 (the Effective Date), the Company entered into a license agreement (the License Agreement) with Ono Pharmaceutical Co., Ltd., a corporation organized and existing under the laws of Japan (Ono), pursuant to which the Company granted Ono exclusive rights to develop and commercialize, at its own cost, selinexor (KPT-330), the Company s lead, novel, oral SINE compound, as well as eltanexor (KPT-8602), the Company s second-generation oral SINE compound, for the diagnosis, treatment and/or prevention of all human oncology indications (the Field) in Japan, Republic of Korea, Republic of China (Taiwan) and Hong Kong, as well as in the ten Southeast Asian countries currently comprising the Association of Southeast Asian Nations (the Ono Territory) (the Exclusive License). Pursuant to the terms of the License Agreement, the Company received an upfront payment of

¥2.5 billion (US$21,916 on the date received), and could receive up to ¥10.15 billion (approximately US$90,500 at the exchange rate as of the Effective Date) in milestone payments if certain development goals are achieved and up to ¥9.0 billion (approximately US$80,200 at the exchange rate as of the Effective Date) in milestone payments if certain sales milestones are achieved, as well as a low double-digit royalty based on future net sales of selinexor and eltanexor in the Ono Territory. In addition, upon Ono s election and the parties full execution of a manufacturing technology transfer plan and satisfaction of other specified conditions (the Manufacturing Election), the Company will grant to Ono non-exclusive rights to manufacture selinexor, eltanexor and products containing such compounds in or outside of the Ono Territory solely for development and commercialization in the Field in the Ono Territory.

As part of the License Agreement, Ono will also have the right to participate in global clinical studies of selinexor and eltanexor, and will bear the cost and expense for patients enrolled in clinical studies in the Ono Territory. Ono is responsible for seeking regulatory and marketing approvals for selinexor and eltanexor in the Ono Territory, as well as any development of the products specifically necessary to obtain such approvals. Ono is also responsible for the commercialization of products containing selinexor or eltanexor in the Field in the Ono Territory at its own cost and expense.

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Subject to Ono s Manufacturing Election, the Company will furnish clinical supplies of drug substance to Ono for use in Ono s development efforts pursuant to a clinical supply agreement to be entered into by the Company and Ono, and Ono may elect to have the Company provide commercial supplies of drug product to Ono pursuant to a commercial supply agreement to be entered into by the Company and Ono, in each case the costs of which will be borne by Ono.

The License Agreement will continue in effect on a product-by-product, country-by-country basis until the later of the tenth anniversary of the first commercial sale of the applicable product in such country or the expiration of specified patent protection and regulatory exclusivity periods for the applicable product in such country. However, the License Agreement may be terminated earlier by (i) either party for breach of the License Agreement by the other party or in the event of the insolvency or bankruptcy of the other party, (ii) Ono on a product-by-product basis for certain safety reasons or on a product-by-product, country-by-country basis for any reason with 180 days prior notice or (iii) the Company in the event Ono challenges or assists with a challenge to certain of the Company s patent rights.

The Company assessed this arrangement in accordance with ASC 606 and concluded that the contract counterparty, Ono, is a customer. The Company identified the following material promises under the contract: (i) Exclusive Licenses for selinexor and eltanexor, (ii) Initial Data Transfer for selinexor and eltanexor, which consisted of regulatory data compiled by the Company for the licensed compounds and products as of the Effective Date, (iii) Initial Clinical Supply for selinexor, which consisted of units of clinical supply for Ono to conduct its Phase I Trial, and (iv) an obligation to stand-ready to provide Initial Clinical Supply for eltanexor. The Company also identified several immaterial promises under the contract relating to information exchanges, and participation on operating committees and other working groups. Separately, the Company also identified certain customer options that would create an obligation for the Company if exercised by Ono, including the (i) Additional Data Transfer for selinexor and eltanexor, which would consist of the transfer of additional regulatory data compiled by the Company for the licensed compounds and products after the Effective Date, (ii) Additional Clinical Supply and Related Substance Supply for selinexor and eltanexor, which would consist of supplying Ono with units and substance of selinexor and eltanexor incremental to the Initial Clinical Supply for selinexor and the obligation to stand-ready to provide Initial Clinical Supply for eltanexor, as noted above, (iii) Manufacturing Technology Transfer and License for selinexor and eltanexor under Ono s Manufacturing Election, as detailed above, and (iv) Option for Backup Compound, which represents Ono s option to select a replacement compound in the event it elects to discontinue to development of either of the licensed compounds. Collectively, these options are referred to herein as the Transfer Option. The Transfer Options individually represent material rights, as they were offered at a significant and incremental discount. Therefore, they were further assessed as performance obligations under the License Agreement. The Company also identified certain other customer options that would create a manufacturing obligation for the Company if exercised by Ono, including commercial supply. This option is referred to herein as the Manufacturing Option. The Manufacturing Option does not represent a material right, as it is not offered at a significant and incremental discount.

In further evaluating the promises detailed above, the Company determined that the (i) Exclusive License, Initial Data Transfer, and Initial Clinical Supply for selinexor and (ii) Exclusive License, Initial Data Transfer, and obligation to stand-ready to provide Initial Clinical Supply of eltanexor were not distinct from one another, and must be combined as two separate performance obligations (the Combined License Obligation for selinexor and Combined License Obligation for eltanexor). This is because, for both selinexor and eltanexor, Ono requires the Initial Data Transfer and clinical supply to derive benefit from the Exclusive Licenses since the Company did not grant manufacting licenses for selinexor and eltanexor at contract inception. The Company also determined that each of the Transfer Options represents a distinct performance obligation. Based on these determinations, the Company identified six distinct performance obligations at the inception of the License Agreement, including (i) the Combined License Obligation for selinexor, (ii) the Combined License Obligation for eltanexor, and the four components of the Transfer Options, including (iii) the material right for Additional Data Transfer, (iv) the material right for Additional Clinical Supply

and Related Substance Supply, (iv) the material right for Manufacturing Technology Transfer and License, and (vi) the material right for the Option for a Backup Compound.

The Company further determined the up-front payment of ¥2.5 billion (US$21,916 on the date received) constituted the entirety of the consideration to be included in the transaction price at contract inception, which was allocated to the performance obligations based on the Company s best estimate of their relative stand-alone selling prices. The Company determined that substantially all of the value in the arrangement is through the Combined License Obligation for selinexor and Combined License Obligation for eltanexor. In connection therewith, the Company estimated the standalone selling price for each of the material rights within the Transfer Options, and determined such amounts were insignificant, and, therefore, immaterial for purposes of allocation. Accordingly, the Company allocated the ¥2.5 billion (US$21,916 on the date received) upfront transaction price amongst the Combined License Obligations as follows: $19,724 for selinexor and $2,192 for eltanexor. The Company believes that a change in the assumptions used to determine its best estimate of the stand-alone selling prices for any of the identified performance obligations would not have a significant effect on the allocation of the underlying transaction price to the performance obligations.

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Upon execution of the Ono Agreement, the transaction price included only the ¥2.5 billion (US$21,916 on the date received) up-front payment owed to the Company. As referenced above, the Company is eligible to receive additional payments of up to ¥10.15 billion based on the achievement by Ono of future specified development milestones and up to ¥9.0 billion based on the achievement by Ono of future specified commercial milestones, as well as a low double-digit royalty based on future net sales of selinexor and eltanexor in the Ono Territory. In addition, the Company could receive cost reimbursement in connection with its promise to stand-ready to provide Initial Clinical Supply for eltanexor in the future. The future regulatory milestones and cost reimbursement for providing Initial Clinical Supply of eltanexor, both of which represent variable consideration, were evaluated under the most likely amount method, and were not included in the transaction price, because the amounts were fully constrained as of March 31, 2018. As part of its evaluation of the constraint, the Company considered numerous factors, including that receipt of such amounts is outside the control of the Company. Separately, any consideration related to sales-based milestones, as well as royalties on net sales upon commercialization by Ono, will be recognized when the related sales occur, as they were determined to relate predominantly to the intellectual property granted to Ono and, therefore, have also been excluded from the transaction price in accordance with the sales-based royalty exception, as well as the Company s accounting policy. The Company will re-evaluate the transaction price in each reporting period, as uncertain events are resolved, or as other changes in circumstances occur.

To date, the Company recognized no revenue associated with this agreement. Revenue will be recognized for (i) the Combined License Obligation for selinexor once the Initial Clinical Supply is delivered, and (ii) the Combined License Obligation for eltanexor once the Company s promise to stand-ready to provide Initial Clinical Supply of eltanexor in the future is fulfilled, which are the only undelivered items in the Combined License Obligation for selinexor and Combined License Obligation for eltanexor, respectively. As of March 31, 2018, the ¥2.5 billion (US$21,916 on the date received) upfront payment represents a contract liability, (i) US$19,724 of which was included in deferred revenue and is classified as a current liability in the condensed consolidated balance sheet and (ii) $2,192 of which was included in deferred revenue and is classified as a non-current liability in the condensed consolidated balance sheet. The Initial Clinical Supply of selinexor was fully delivered during the quarter ending June 30, 2018, and the transaction price allocated to the Combined License Obligation for selinexor, or US$19,724, will be recognized in that period.

Given the determination that the license rights conveyed to Ono lacked standalone value from the initial clinical supply of product required for Ono to obtain benefit from the rights granted and the fact that no initial clinical supply had been provided to Ono as of December 31, 2017, the Company concluded that no revenue should be recognized under ASC 605. Arrangement consideration at the inception of the arrangement included the ¥2.5 billion (US$21,916 on the date received) upfront payment. All other forms of consideration such as milestones and royalties, were considered contingent consideration, with no amount allocable to deliverables at the inception of the arrangement. The Company concluded the contingent consideration would be recognized when the underlying contingencies have been resolved, assuming all other revenue recognition criteria are met. As the accounting treatment for this agreement did not materially differ under ASC 605 and ASC 606, and no revenue was recognized under the Company s previous accounting policy through December 31, 2017, no transition adjustment was recorded to the opening balance of accumulated deficit as of January 1, 2018. Accordingly, the upfront payment of¥2.5 billion (US$21,916 on the date received), which again represents a contract liability, was also included in deferred revenue as of December 31, 2017.

MMRF Research Agreement

The Company is a party to a research agreement with the Multiple Myeloma Research Foundation (MMRF). Under this research agreement, the Company is obligated to make certain payments to MMRF, including if the Company out-licenses selinexor. The terms of this research agreement do not apply to eltanexor. In connection with the transactions contemplated under the Agreement, the Company paid to MMRF approximately ¥225 million

(US$1,972) of the upfront cash payment from Ono in the year ended December 31, 2017, and it will be obligated to pay a percentage of any milestone payments from Ono and a mid-single-digit percentage of any royalty payments from Ono. Such payments are recorded within research and development expense in the Company s condensed consolidated statement of operations. The maximum aggregate amount the Company may be obligated to pay to MMRF under the research agreement is $6,000.

Anivive License Agreement

On April 28, 2017, the Company entered into a license agreement with Anivive Lifesciences, Inc. (Anivive), a biopharmaceutical company engaged in the research, development and commercialization of animal health medicines, pursuant to which the Company has granted Anivive an exclusive, worldwide license to develop and commercialize verdinexor (KPT-335) for the treatment of cancer in companion animals (the Anivive Agreement) (the Exclusive License). Pursuant to the terms of the Anivive Agreement, the Company received an upfront payment of $1,000 and a payment of $250 upon the completion of the technology transfer, which occurred during the year ended December 31, 2017. In addition, the Company is eligible to receive potential clinical, regulatory and commercial development milestone payments totaling up to $43,250, as well as a low double-digit royalty based on Anivive s future net sales of verdinexor following commercialization. The potential future milestone payments are composed of $5,750 based on achievement of clinical and regulatory milestone events and $37,500 based on achievement of sales milestone events.

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The Company assessed this arrangement in accordance with ASC 606 and concluded that the contract counterparty, Anivive, is a customer. The Company identified the following material promises under the contract, the Exclusive License and the Technology Transfer, which consisted of regulatory data compiled by the Company for the licensed compound and product as of the Effective Date. The Company also identified the following immaterial promises under the contract that were not deemed performance obligations, including participating on a product advisory committee and sharing regulatory matter information. The Company further determined other promises for (i) transfer of additional technology in the future, if developed by the Company, and (ii) facilitating manufacturing and supply relationships with the Company s third-party contract manufacturers represented customer options, which would create an obligation for the Company if exercised by Anivive. Since either no additional or immaterial consideration is owed to the Company by Anivive upon exercise of the customer options noted, the Company determined both are offered at significant and incremental discounts. Accordingly, they were assessed as material rights and, therefore, separate performance obligations in the arrangement.

In further evaluating the promises detailed above, the Company determined that the Exclusive License and Technology Transfer were not distinct from one another and must be combined as a performance obligation (the Combined License Obligation). This is because Anivive requires the Technology Transfer to derive benefit from the Exclusive License. Based on these determinations, the Company identified three distinct performance obligations at the inception of the contract: (i) the Combined License Obligation, (ii) the material right for transfer of additional technology in the future, if developed by the Company, and (iii) the material right for facilitating manufacturing and supply relationships with the Company s third-party contract manufacturers.

The Company further determined that the up-front payment of $1,000 upon contract execution, as well as the $250 upon completion of the Technology Transfer, constituted the entirety of the consideration to be included in the transaction price as of the transition date, January 1, 2018, which was allocated to the performance obligations based on their relative stand-alone selling prices. In connection therewith, the Company estimated the stand-alone selling price of the (i) Combined License Obligation, (ii) material right for transfer of additional technology in the future, if developed by the Company, and (iii) the material right for facilitating manufacturing and supply relationships with the Company s third-party contract manufacturers, and determined the stand-alone selling price of the material rights noted were insignificant based on various qualitative considerations. Accordingly, the Company further determined the allocation of the upfront payment to the material rights noted was insignificant. Based on the estimates of the stand-alone selling prices for each of the performance obligations, the Company determined substantially all the $1,250 transaction price should be allocated to the Combined License Obligation. The Company believes that a change in the assumptions used to determine its best estimate of the stand-alone selling prices for the identified performance obligations would not have a significant effect on the allocation of the underlying transaction price to the performance obligations.

As referenced above, the up-front payment of $1,000 upon contract execution, as well as the $250 upon completion of the Technology Transfer, constituted the entirety of the consideration to be included in the transaction price as of the transition date, January 1, 2018. The Company is also eligible to receive additional payments up to $5,750 based on achievement of clinical and regulatory milestone events and up to $37,500 based on achievement of sales milestone events, as well as a low double-digit royalty based on Anivive s future net sales of verdinexor following commercialization. The future regulatory milestones, which represent variable consideration, were evaluated under the most likely amount method, and were not included in the transaction price, because the amounts are fully constrained as of March 31, 2018. As part of its evaluation of the constraint, the Company considered numerous factors, including that receipt of such milestones is outside the control of the Company. Separately, any consideration related to sales-based milestones, as well as royalties on net sales upon commercialization by Anivive, will be recognized when the related sales occur, as they were determined to relate predominantly to the intellectual property granted to Anivive and, therefore, have also been excluded from the transaction price in accordance with the sales-based royalty

exception, as well as the Company s policy. The Company will re-evaluate the transaction price in each reporting period, as uncertain events are resolved, or as other changes in circumstances occur.

To date, the Company recognized $1,250 of revenue associated with the Anivive Agreement. Revenue for the upfront payment and technology transfer milestone was recognized upon completion of the Technology Transfer in October 2017, as all promises under the Combined License Obligation had been fulfilled.

The Company reached similar conclusions when evaluating this agreement under its previous accounting policy, which was based on legacy guidance within ASC 605. When evaluating this agreement under ASC 605, the Company concluded that the licenses to verdinexor and technology transfer concerning the licensed product are essential to Anivive s intended use of the license to develop and commercialize the Licensed Compounds and represented a single unit of accounting. Other potential contractual obligations were evaluated and determined not to be deliverables at inception of the arrangement or were evaluated and determined to be immaterial to the arrangement and, therefore, not evaluated further in the Company s analysis. Arrangement consideration at the inception of the arrangement included the $1,250 in upfront payments, which includes the milestone fee upon completion of the Technology Transfer. All other forms of consideration, such as milestones and royalties, were considered contingent consideration, with no amount allocable

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to deliverables at the inception of the arrangement. The Company concluded the contingent consideration would be recognized when the underlying contingencies have been resolved, assuming all other revenue recognition criteria are met. Given the single unit of accounting and that the Technology Transfer would be the last item to be delivered within the unit of accounting, the Company concluded that revenue would be recognized upon the completion of delivery of the technology transfer assuming all other general revenue recognition criteria would be met as of that date. As the accounting treatment for this agreement did not materially differ under ASC 605 and ASC 606, and the upfront payment and technology transfer fee, totaling $1,250, was recognized as revenue during the year ended December 31, 2017 in accordance with the Company s previous accounting policy, and would have also been recognized during the year ended December 31, 2017 in accordance with the Company s accounting policy under ASC 606, no transition adjustment was recorded to the opening balance of accumulated deficit as of January 1, 2018.

4. Fair Value of Financial Instruments

Financial instruments, including cash, restricted cash, prepaid expenses and other current assets, accounts payable and accrued expenses are presented in the condensed consolidated financial statements at amounts that approximate fair value at March 31, 2018 and December 31, 2017.

The Company is required to disclose information on all assets and liabilities reported at fair value that enables an assessment of the inputs used in determining the reported fair values. The fair value hierarchy 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
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 classified as Level 2 within the valuation hierarchy consist of commercial paper, corporate debt securities, U.S. government agency securities and certificates of deposit. The Company estimates the fair values of these marketable securities by taking into consideration valuations obtained from third-party pricing sources. These pricing sources utilize industry standard valuation models, including both income and market-based approaches, for which all significant inputs are observable, either directly or indirectly, to estimate fair value. These inputs include market pricing based on real-time trade data for the same or similar securities, issuer credit spreads, benchmark yields, and other observable inputs. The Company validates the prices provided by its third-party pricing sources by understanding the models used, obtaining market values from other pricing sources and analyzing pricing data in certain instances.

The following table presents information about the Company s financial assets that have been measured at fair value at March 31, 2018 and indicates the fair value hierarchy of the valuation inputs utilized to determine such fair value (in thousands):

Description	**Total**

		Quoted Prices in Active Markets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Financial assets				
Cash equivalents:				
Money market funds	$ 15,551	$ 15,551	$	$
Investments:				
Current:				
Corporate debt securities	79,857		79,857	
Commercial paper	6,074		6,074	
U.S. government and agency securities	4,987		4,987	
Certificates of deposit	2,500		2,500	
Non-current:				
Corporate debt securities (one to two year maturity)	7,836		7,836	
U.S. government and agency securities	2,478		2,478	
	$ 119,283	$ 15,551	$ 103,732	$

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The following table presents information about the Company s financial assets that have been measured at fair value at December 31, 2017 and indicates the fair value hierarchy of the valuation inputs utilized to determine such fair value (in thousands):

Description	Total	Quoted Prices in Active Markets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Financial assets				
Cash equivalents:				
Money market funds	$ 41,805	$ 41,805	$	$
Investments:				
Current:				
Corporate debt securities	66,253		66,253	
Commercial paper	6,720		6,720	
Certificates of deposit	2,500		2,500	
U.S. government and agency securities	1,999		1,999	
Non-current:				
Corporate debt securities (one to two year maturity)	26,916		26,916	
U.S. government securities and agency securities	2,480		2,480	
	$ 148,673	$ 41,805	$ 106,868	$

5. Investments

The following table summarizes the Company s investments as of March 31, 2018 (in thousands):

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Loss	Fair Value
Current:				
Corporate debt securities	$ 80,124	$ 2	$ (269)	$ 79,857
Commercial paper	6,074			6,074
U.S. government and agency securities	4,989		(2)	4,987
Certificates of deposit	2,500			2,500
Non-current:				
Corporate debt securities (one to two year maturity)	7,907		(71)	7,836
U.S. government and agency securities	2,500		(22)	2,478
	$ 104,094	$ 2	$ (364)	$ 103,732

The following table summarizes the Company s investments as of December 31, 2017 (in thousands):

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Loss	Fair Value
Current:				
Corporate debt securities	$ 66,384	$	$ (131)	$ 66,253
Commercial paper	6,719	1		6,720
Certificates of deposit	2,500			2,500
U.S. government and agency securities	2,000		(1)	1,999
Non-current:				
Corporate debt securities (one to two year maturity)	27,018	2	(104)	26,916
U.S. government and agency securities	2,500		(20)	2,480
	$ 107,121	$ 3	$ (256)	$ 106,868

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At March 31, 2018 and December 31, 2017, the Company held 49 and 54 debt securities, respectively, that were in an unrealized loss position for less than one year. The aggregate fair value of debt securities in an unrealized loss position at March 31, 2018 and December 31, 2017 was $91,549 and $96,623, respectively. There were no individual securities that were in a significant unrealized loss position or that had been in an unrealized loss position for greater than one year as of March 31, 2018 or December 31, 2017.

The Company reviews investments for other-than-temporary impairment whenever the fair value of an investment is less than the amortized cost and evidence indicates that an investment s carrying amount is not recoverable within a reasonable period of time. Other-than-temporary impairments of investments are recognized in the condensed consolidated statements of operations if the Company has experienced a credit loss and has the intent to sell the investment or if it is more likely than not that the Company will be required to sell the investment before recovery of the amortized cost basis. Evidence considered in this assessment includes reasons for the impairment, compliance with the Company s investment policy, the severity and the duration of the impairment and changes in value subsequent to the end of the period.

6. Property and Equipment, net

Property and equipment, net consisted of the following (in thousands):

	Estimated Useful Life Years	March 31, 2018	December 31, 2017
Laboratory equipment	4	$ 593	$ 593
Furniture and fixtures	5	381	381
Office and computer equipment	3	378	378
Construction in process	N/A	438	
Leasehold improvements	Lesser of useful life or lease term	3,391	3,391
		5,181	4,743
Less accumulated depreciation and amortization		(2,727)	(2,558)
		$ 2,454	$ 2,185

7. Accrued Expenses

Accrued expenses consisted of the following (in thousands):

	March 31, 2018	December 31, 2017
Research and development costs	$ 16,978	$ 16,198
Payroll and employee-related costs	2,518	3,982
Professional fees	1,764	972

Other	285	293
	$ 21,545	$ 21,445

8. Net Loss Per Share

Basic and diluted net loss per common share is calculated by dividing net loss 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 unvested restricted stock and restricted stock units, 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 due to their anti-dilutive effect:

	Three Months Ended March 31,	
	2018	**2017**
Outstanding stock options	8,702,552	6,867,142
Unvested restricted stock units	228,100	462,250

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9. Stock-based Compensation

Stock Options

A summary of the Company s stock option activity and related information follows:

	Shares	Weighted-Average Exercise Price Per Share	Weighted-Average Remaining Contractual Term (years)	Aggregate Intrinsic Value (in thousands)
Outstanding at December 31, 2017	7,019,083	$ 13.77	7.4	$ 11,897
Granted	2,184,200	11.04		
Exercised	(132,178)	3.26		
Canceled	(368,553)	14.65		
Outstanding at March 31, 2018	8,702,552	$ 13.21	7.8	$ 31,741
Exercisable at March 31, 2018	4,038,071	$ 15.51	6.1	$ 17,903

Total stock-based compensation expense related to stock options for the three months ended March 31, 2018 and 2017 was $3,918 and $4,939, respectively.

As of March 31, 2018, there was $33,656 of total unrecognized stock-based compensation expense related to stock options. The expense is expected to be recognized over a weighted-average period of 3.06 years.

Restricted Stock Units

A restricted stock unit (RSU) represents the right to receive one share of the Company s common stock upon vesting of the RSU. The fair value of each RSU is based on the closing price of the Company s common stock on the date of grant.

During the year ended December 31, 2017, the Company granted RSUs with service conditions that vest provided that the employee remains employed with the Company (Time-Based RSUs). The following is a summary of Time-Based RSU activity under the 2013 Stock Incentive Plan for the three months ended March 31, 2018:

	Number of Shares Underlying RSUs	Weighted-Average Grant Date Fair Value
Unvested at December 31, 2017	30,000	$ 10.52
Granted		
Forfeited		
Vested	(5,000)	10.27

Unvested at March 31, 2018	25,000	$	10.57

The total stock-based compensation expense related to Time-Based RSUs for the three months ended March 31, 2018 and 2017 was $39 and $917, respectively. As of March 31, 2018, there was $213 of unrecognized compensation costs related to unvested Time-Based RSUs, which are expected to be recognized over a weighted-average period of 1.4 years.

Separately, and during the year ended December 31, 2017, the Company granted performance-based RSUs, which vest upon the achievement of certain performance goals subject to the employee s continued employment (Performance-Based RSUs). The grant date fair value of the outstanding Performance-Based RSUs is $2,400 as of March 31, 2018, and will be recognized on an accelerated attribution basis when the Performance-Based RSUs are deemed probable of achievement to the date the awards vest. During the three months ended March 31, 2018, the Company recognized $137 of stock-based compensation expense related to the Performance-Based RSUs, as the performance goal related to certain Performance-Based RSUs was deemed probable of achievement as of March 31, 2018 and was achieved on April 28, 2018. However, as of March 31, 2018, the 203,100 outstanding awards were still unvested.

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Employee Stock Purchase Plan

The Company has an Employee Stock Purchase Plan (ESPP) that permits eligible employees to enroll in six-month offering periods. Participants may purchase shares of the Company s common stock, through payroll deductions, at a price equal to 85% of the fair market value of the common stock on the first or last day of the applicable six-month offering period, whichever is lower. Purchase dates under the ESPP occur on or about May 1 and November 1 of each year. In 2013, the Company s stockholders approved the reservation of 242,424 shares of the Company s common stock for issuance under the ESPP, plus an annual increase to be added on the first day of each fiscal year, commencing on January 1, 2015 and ending on December 31, 2023, equal to the lesser of 484,848 shares of the Company s common stock, 1% of the number of outstanding shares on such date, or an amount determined by the board of directors.

For the three months ended March 31, 2018 and 2017, the Company recorded stock-based compensation expense related to the ESPP of $70 and $53, respectively. As of March 31, 2018, 433,511 shares of the Company s common stock remained available for issuance under the ESPP. As of March 31, 2018, there was $23 of total unrecognized stock-based compensation expense related to the ESPP. The expense is expected to be recognized over a period of one month.

10. Commitments and Contingencies

In March 2014, the Company entered into an operating lease for approximately 29,933 square feet of office and research space in Newton, Massachusetts. The Company uses the leased premises as its corporate headquarters and for research and development purposes, as well as its commercial and administrative requirements. The lease was amended on December 31, 2014 by extending the term of the lease from November 30, 2021 to September 30, 2022. This amendment also provided for the expansion of the premises leased by the Company by approximately 16,234 square feet. The lease was amended again on February 28, 2018 by extending the term of the lease from September 30, 2022 to September 30, 2025. The amendment also provided for the expansion of the premises leased by the Company by approximately 15,976 square feet. The amendment from February 2018 also provided the Company with the right of first offer to lease between 20,000 and approximately 36,000 square feet of additional space.

The Company evaluated the lease amendments and determined that the classification as an operating lease had not changed, and that the amendments did not constitute a new lease. As such, the unamortized balances of the existing deferred rent and tenant improvement allowances, along with the additions to deferred rent and tenant improvement allowances associated with the February 28, 2018 amendment, will be amortized over the term of that lease amendment. The Company is recording rent expense on a straight-line basis through the end of the lease term, inclusive of the period in which there are no scheduled rent payments. The Company has recorded deferred rent on the condensed consolidated balance sheets at March 31, 2018 and December 31, 2017, accordingly. The lease provided the Company with an allowance for improvements of $1,616 which was incurred in the first quarter of 2015. The amended lease provided the Company with an allowance for improvements of up to $731, of which $438 was incurred in the first quarter of 2018. All improvements were deemed normal tenant improvements, were recorded as leasehold improvements and deferred rent and will be recorded as a reduction to rent expense ratably over the lease term. The Company evaluated the lease amendments and determined they did not constitute a new lease and the accounting treatment noted was appropriate. The Company has provided a security deposit in the form of a cash-collateralized letter of credit in the amount of $400, which amount was reduced to $200 in January 2018. The amount is classified as non-current restricted cash on the condensed consolidated balance sheet.

In November 2014, the Company signed a five-year operating lease agreement in Munich, Germany for approximately 3,681 square feet of office space. The lease is for the period February 2015 through January 2020. Pursuant to the lease agreement, the Company was obligated to make aggregate rent payments of 374 (approximately

US$461) through January 31, 2020. The Company is recording rent expense on a straight-line basis through the end of the lease term, inclusive of the period in which there are no scheduled rent payments.

The Company recorded rent expense totaling $373 and $301 for the three months ended March 31, 2018 and 2017, respectively.

11. Equity

Controlled Equity Offering Sales Agreement

On December 7, 2015, the Company entered into a Controlled Equity Offering Sales Agreement (as amended from time to time, the Sales Agreement) with Cantor Fitzgerald & Co., as sales agent (Cantor), pursuant to which the Company issued and sold through Cantor, shares of the Company s common stock (the Shares) with an aggregate offering price of $50,000. On November 7, 2016, the Company entered into an amendment to the Sales Agreement pursuant to which the Company issued and sold Shares with

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an additional aggregate offering price of $50,000. On December 1, 2017, the Company entered into a second amendment to the Sales Agreement that provides that it may issue and sell Shares having an additional aggregate offering price of up to $75,000.

Under the Sales Agreement, Cantor may sell the Shares by methods deemed to be an at-the-market offering as defined in Rule 415 promulgated under the Securities Act of 1933, as amended (the Securities Act), including sales made directly on The Nasdaq Global Select Market, on any other existing trading market for the Shares or to or through a market maker. In addition, under the Sales Agreement, Cantor may sell the Shares by any other method permitted by law.

The Company is not obligated to make any sales of the Shares under the Sales Agreement. The Company or Cantor may suspend or terminate the offering of Shares upon notice to the other party and subject to other conditions. The Company will pay Cantor a commission of up to 3.0% of the gross proceeds from the sale of the Shares pursuant to the Sales Agreement and has agreed to provide Cantor with customary indemnification and contribution rights.

As of May 1, 2018, the Company had sold an aggregate of 9,172,159 Shares under the Sales Agreement, for net proceeds of approximately $89,053. The Company sold no Shares under the Sales Agreement during the three months ended March 31, 2018.

12. Subsequent Event

On May 7, 2018, the Company completed a follow-on offering under its shelf registration statement on Form S-3 (File No. 333-222726) pursuant to which the Company issued an aggregate of 10,525,424 shares of common stock, which included the full exercise of the underwriters option to purchase additional shares, at a public offering price of $14.75 per share. The Company received aggregate net proceeds of approximately $145,635 from the offering after deducting the underwriting discounts and commissions and other estimated offering expenses.

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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 financial statements and related notes appearing elsewhere in this quarterly report.

FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q, including the following discussion, contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this Quarterly Report on Form 10-Q, including statements regarding possible achievement of discovery and development milestones, our future discovery and development efforts, our collaborations and partnering agreements with third parties, our strategy, our future operations, financial position and revenues, projected costs, prospects, plans and objectives of management, are forward looking statements. The words anticipate, believe, estimate, expect, intend may, plan, predict, project, target, potential, will, would, could, should, continue and similar e 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 plans, intentions, expectations or results discussed in the forward-looking statements. Factors that could cause actual results to differ materially from those in the forward-looking statements include, but are not limited to, adverse results in our drug discovery and clinical development activities, decisions made by the U.S. Food and Drug Administration (FDA) and other regulatory authorities with respect to the development and commercialization of our drug candidates, our ability to raise additional capital to support our clinical development program and other operations, our ability to develop products of commercial value and to identify, discover and obtain rights to additional potential product candidates, our ability to obtain, maintain and enforce our intellectual property, the outcome of research and development activities and the fact that the preclinical and clinical testing of our compounds may not be predictive of the success of later clinical trials, our reliance on third-parties, competitive developments, the effect of current and future legislation and regulation and regulatory actions, as well as other risks described in this Quarterly Report on Form 10-Q, our Annual Report on Form 10-K for the year ended December 31, 2017 (2017 Form 10-K), as filed with the Securities and Exchange Commission (SEC), on March 15, 2018, and other filings with the SEC.

As a result of these and other factors, we may not actually achieve the plans, intentions, expectations or results 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. We do not assume any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by law.

OVERVIEW

We are a clinical-stage pharmaceutical company focused on the discovery, development and subsequent commercialization of novel, first-in-class drugs directed against nuclear transport and related targets for the treatment of cancer and other major diseases. Our scientific expertise is focused on understanding the regulation of intracellular communication between the nucleus and the cytoplasm. We have discovered and are developing wholly-owned, novel, small molecule **S**elective **I**nhibitor of **N**uclear **E**xport (**SINE**) compounds that inhibit the nuclear export protein exportin 1 (XPO1). These SINE compounds represent a new class of drug candidates with a novel mechanism of action that have the potential to treat a variety of diseases in areas of unmet medical need. Our SINE compounds were the first oral XPO1 inhibitors in clinical development.

Our focus is on seeking the regulatory approval and commercialization of our lead drug candidate, selinexor (KPT-330), as an oral agent in cancer indications with significant unmet clinical need, initially for hematologic malignancies. We then plan to seek additional approvals for the use of selinexor in combination therapies to expand the patient populations that are eligible for selinexor, as well as to move selinexor towards front-line cancer therapy. We are also advancing the clinical development of selinexor in multiple solid tumor indications. To date, over 2,400 patients have been treated with oral selinexor in company- and investigator-sponsored clinical trials in advanced hematologic malignancies and solid tumors. Clinical trials evaluating selinexor include the Phase 2b STORM (**S**elinexor **T**reatment **o**f **R**efractory **M**yeloma) study in multiple myeloma, the Phase 1b/2 STOMP (**S**elinexor and Backbone **T**reatments **o**f **M**ultiple Myeloma **P**atients) study in combination with standard therapies in multiple myeloma, the Phase 2b SADAL (**S**elinexor **A**gainst **D**iffuse **A**ggressive **L**ymphoma) study in diffuse large B-cell lymphoma (DLBCL), the pivotal, randomized Phase 3 BOSTON (**Bo**rtezomib, **S**elinexor and Dexame**t**has**on**e) study in multiple myeloma, and the Phase 2/3 SEAL (**Se**linexor in **A**dvanced **L**iposarcoma) study in liposarcoma.

We recently reported top-line data from the expanded cohort for the STORM study on April 30, 2018 and expect to provide top-line data from the SADAL study by the end of 2018, top-line data from the BOSTON study in 2019 and top-line data from the Phase 3

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portion of the SEAL study by the end of 2019. We are also establishing the commercial infrastructure to support a potential launch of selinexor in the United States and we intend to work with existing and potential partners to establish such commercial infrastructure outside the United States. To date, we have financed our operations principally through private placements of our preferred stock, proceeds from our initial public offering and follow-on offerings of common stock and cash generated from our business development activities.

As of March 31, 2018, we had an accumulated deficit of $533.8 million. We had net losses of $38.5 million and $29.9 million for the three months ended March 31, 2018 and 2017, respectively. We have not generated any revenue to date from the sales of any drugs.

We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. The net losses we incur may fluctuate significantly from quarter to quarter. We anticipate that our expenses will increase substantially if and as we:

- continue our research and preclinical and clinical development of our drug candidates;
- initiate additional clinical trials for our drug candidates;
- seek marketing approvals for any of our drug candidates that successfully complete clinical trials;
- establish a sales, marketing and distribution infrastructure to commercialize any drugs for which we may obtain marketing approval;
- maintain, expand and protect our intellectual property portfolio;
- manufacture our drug candidates;
- hire additional clinical, quality control and scientific personnel;
- identify additional drug candidates;
- acquire or in-license other drugs and technologies; and
- add operational, financial and management information systems and personnel, including personnel to support our drug development, any future commercialization efforts and our other operations as a public company.

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.

There were no changes to the critical accounting policies we identified in the 2017 Form 10-K, other than the adoption of ASU No. 2014-09, as described further in Note 1 to the Condensed Consolidated Financial Statements. It is important that the discussion of our operating results that follows be read in conjunction with the critical accounting policies disclosed in the 2017 Form 10-K.

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RESULTS OF OPERATIONS

Comparison of the Three Months Ended March 31, 2018 and March 31, 2017

	Three Months Ended March 31,			
	2018	2017	$ Change	% Change
	(in thousands)			
License and other revenue	$ 10,000	$ 68	$ 9,932	14,605.9%
Operating expenses:				
Research and development	41,321	24,083	17,238	71.6%
General and administrative	7,621	6,264	1,357	21.7%
Loss from operations	(38,942)	(30,279)	(8,663)	28.6%
Other income, net	495	385	110	28.6%
Loss before income taxes	(38,447)	(29,894)	(8,553)	28.6%
Provision for income taxes	(12)	(23)	11	(47.8)%
Net loss	$ (38,459)	$ (29,917)	$ (8,542)	28.6%

License and Other Revenue. We recognized revenue pursuant to an Asset Purchase Agreement, or APA, with Biogen MA Inc., or Biogen, in 2018 and pursuant to a government grant in 2017. Revenue for the three months ended March 31, 2018 was $10.0 million compared to $0.1 million for the three months ended March 31, 2017. The increase in revenue during the three months ended March 31, 2018 was primarily the result of entering into the APA with Biogen in January 2018 and the satisfaction of the related revenue recognition criterion, which resulted in revenue of $10.0 million.

Research and Development Expense. Research and development expense increased approximately $17.2 million to $41.3 million for the three months ended March 31, 2018 from approximately $24.1 million for the three months ended March 31, 2017. The increase is primarily related to:

- an increase of $10.3 million in clinical trial costs, primarily related to the selinexor program;
- an increase of $2.2 million in consulting and professional expense;
- an increase of $1.8 million in personnel costs, primarily due to increased headcount and related onboarding costs;
- an increase of $1.6 million related to our obligation to pay a portion of upfront fees received from the APA with Biogen to a third party;

an increase of $0.9 million in costs related to our focus on a New Drug Application (NDA), and discovery and travel costs; and

an increase of $0.4 million in other costs.

We expect our research and development expenses to continue to increase for the full year 2018 compared with the prior year as we continue spending on our development programs and clinical trials, including the continued clinical development of selinexor in our lead indications with a focus on regulatory submissions for selinexor. We plan to submit a New Drug Application , or NDA, to the Food and Drug Administration, or FDA, during the second half of 2018, with a request for accelerated approval for selinexor as a new treatment for patients with penta-refractory multiple myeloma as a result of our positive outcome from the expanded cohort of the STORM study. We also plan to submit a Marketing Authorization Application to the European Medicines Agency in early 2019 with a request for conditional approval.

General and Administrative Expense. General and administrative expense increased approximately $1.4 million to $7.6 million for the three months ended March 31, 2018 from approximately $6.3 million for the three months ended March 31, 2017. The increase is primarily related to:

an increase in consulting and professional costs of $0.9 million;

an increase of $0.2 million in occupancy costs; and

an increase of $0.3 million in other costs.

We expect general and administrative expenses to increase in the future in support of our expanding operating and commercial activities.

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Other Income, net. Other income, net, increased approximately $0.1 million to $0.5 million for the three months ended March 31, 2018 from approximately $0.4 million for the three months ended March 31, 2017. The increase is primarily due to increased investment returns resulting from a general increase in interest rates.

LIQUIDITY AND CAPITAL RESOURCES

Sources of Liquidity

To date, we have not generated any material revenues. We have financed our operations to date principally through private placements of our preferred stock, proceeds from public offerings of our common stock and cash generated from our business development activities.

As of March 31, 2018, we had $141.2 million in cash, cash equivalents and short- and long-term investments compared to $175.9 million in cash, cash equivalents and short- and long-term investments as of December 31, 2017.

In December 2015, we entered into a sales agreement (as amended from time to time, the Sales Agreement), relating to an at-the-market offering, pursuant to which we issued and sold shares of our common stock with an aggregate offering price of $50.0 million. On November 7, 2016, we entered into an amendment to the Sales Agreement pursuant to which we issued and sold shares of our common stock with an additional aggregate offering price of $50.0 million. On December 1, 2017, we entered into a second amendment to the Sales Agreement pursuant to which we may issue and sell shares of our common stock having an additional aggregate offering price of up to $75.0 million on or after December 1, 2017. As of March 31, 2018, we had sold an aggregate of 9,172,159 shares pursuant to this at-the-market offering, for net proceeds of approximately $89.1 million. There have been no sales pursuant to this at-the-market offering during 2018.

On May 7, 2018, we completed a follow-on offering under our shelf registration statement on Form S-3 (File No. 333-222726) pursuant to which we issued an aggregate of 10,525,424 shares of common stock, which included the full exercise of the underwriters option to purchase additional shares, at a public offering price of $14.75 per share. We received aggregate net proceeds of approximately $145.6 million from the offering after deducting the underwriting discounts and commissions and other estimated offering expenses.

On October 11, 2017 (Effective Date), we entered into a license agreement (License Agreement), with Ono Pharmaceutical Co., Ltd., a corporation organized and existing under the laws of Japan (Ono), pursuant to which we granted Ono exclusive rights to develop and commercialize, at its own cost, selinexor and eltanexor for the diagnosis, treatment and/or prevention of all human oncology indications, or Field, in Japan, Republic of Korea, Republic of China (Taiwan) and Hong Kong as well as in the ten Southeast Asian countries currently comprising the Association of Southeast Asian Nations (Ono Territory). Pursuant to the terms of the License Agreement, we received an upfront payment of ¥2.5 billion (US$21.9 million on the date received), and could receive up to ¥10.15 billion (US$90.5 million at the exchange rate as of the Effective Date) in milestone payments if certain development goals are achieved and up to ¥9.0 billion (US$80.2 million at the exchange rate as of the Effective Date) in milestone payments if certain sales milestones are achieved, as well as a low double-digit royalty based on future net sales of selinexor and eltanexor in the Ono Territory.

We are a party to a research agreement with the Multiple Myeloma Research Foundation (MMRF). Under this research agreement, we are obligated to make certain payments to MMRF, including payments in the event we out-license selinexor. The terms of this research agreement do not apply to eltanexor. In connection with the transactions contemplated under the License Agreement, we paid to MMRF approximately ¥225 million (approximately US$2.0 million) of the upfront cash payment from Ono, and we are obligated to pay a percentage of

any milestone payments from Ono and a mid-single-digit percentage of any royalty payments from Ono. The maximum aggregate amount we may be obligated to pay to MMRF under the research agreement is $6.0 million.

On January 24, 2018, we entered into an Asset Purchase Agreement (APA), with Biogen MA Inc., a Massachusetts corporation and subsidiary of Biogen, Inc. (Biogen), pursuant to which Biogen acquired exclusive worldwide rights to develop and commercialize our oral SINE compound KPT-350 and certain related assets with an initial focus in amyotrophic lateral sclerosis (ALS).

Under the terms of the APA, Biogen purchased KPT-350 and certain related assets and assumed certain related liabilities. We received a one-time upfront payment of $10.0 million from Biogen and are eligible to receive additional payments of up to $207.0 million based on the achievement by Biogen of future specified development and commercial milestones. We are also eligible to receive tiered royalty payments that reach low double digits based on future net sales until the later of the tenth anniversary of the first commercial sale of the applicable product and the expiration of specified patent protection for the applicable product, determined on a country-by-country basis.

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We expect that our cash, cash equivalents and short- and long-term investments as of March 31, 2018, totaling $141.2 million, in addition to the $145.6 million of net proceeds we raised in the public common stock offering in May 2018, will be sufficient to fund our current operating plans and capital expenditure requirements into the third quarter of 2019 while we are establishing the commercial infrastructure for a potential launch of selinexor in the United States. Our need for additional funds thereafter may be partially offset by: (i) cash generated from sales of drugs if selinexor receives accelerated approval and we successfully commercialize selinexor in the United States and (ii) from potential future payments related to collaboration or license arrangements we may seek to enter into as part of our strategy to commercialize selinexor outside the United States.

Cash Flows

The following table provides information regarding our cash flows:

	Three Months Ended March 31,	
	2018	2017
	(in thousands)	
Net cash used in operating activities	$ (34,652)	$ (24,708)
Net cash provided by investing activities	2,484	549
Net cash provided by financing activities	429	57
Effect of exchange rate changes	43	16
Net increase (decrease) in cash and cash equivalents	$ (31,696)	$ (24,086)

Operating activities. The net cash used in operating activities in both periods resulted primarily from our net losses adjusted for non-cash charges and changes in the components of working capital. The increase in cash used in operating activities during the three months ended March 31, 2018 compared to the three months ended March 31, 2017 was driven primarily by an increase in our net loss due to an increase in our operating expenses, as adjusted for stock-based compensation and offset by an increase of approximately $9.9 million in revenue, which was primarily attributable to the upfront payment of $10.0 million received from the Biogen.

Investing activities. The net cash provided by investing activities during the three months ended March 31, 2018 reflects an increase of $2.0 million primarily related to the increase in proceeds from maturities of investments and a decrease of $0.3 million in purchases of investments offset by an increase of $0.4 million in purchases of property and equipment compared to the three months ended March 31, 2017.

Financing activities. The net cash provided by financing activities for the three months ended March 31, 2018 reflects an increase of $0.4 million related to an increase in proceeds from the exercise of stock options compared to the three months ended March 31, 2017.

Funding Requirements

We expect our expenses to increase in connection with our ongoing activities, particularly as we continue the clinical trials of, and assuming positive results of our clinical trials and based on regulatory feedback, if and when we seek marketing approval for, selinexor and our other drug candidates. In addition, if we obtain marketing approval for any of our drug candidates, we expect to incur significant commercialization expenses related to drug sales, marketing, manufacturing and distribution to the extent that such sales, marketing, manufacturing and distribution are not the

responsibility of any collaborator that we may have at such time for any such drug. Furthermore, we expect to continue to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we would be forced to delay, reduce or eliminate our research and development programs or future commercialization efforts.

We expect that our cash, cash equivalents and short- and long-term investments as of March 31, 2018, totaling $141.2 million, in addition to the $145.6 million of net proceeds we raised in the public common stock offering in May 2018, will be sufficient to fund our current operating and capital expenditure plans into the third quarter of 2019 while we are establishing the commercial infrastructure for a potential launch of selinexor in the United States. Our need for additional funds thereafter may be partially offset by: (i) cash generated from sales of drugs if selinexor receives accelerated approval and we successfully commercialize selinexor in the United States and (ii) from potential future payments related to collaboration or license arrangements we may seek to enter into as part of our strategy to commercialize selinexor outside the United States. However, our future capital requirements will depend on many factors, including:

- the progress and results of our current and planned clinical trials of selinexor;

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- the scope, progress, results and costs of drug discovery, preclinical development, laboratory testing and clinical trials for our other drug candidates;
- the costs, timing and outcome of regulatory review of our drug candidates;
- our ability to establish and maintain collaborations on favorable terms, if at all;
- the success of any collaborations that we may enter into with third parties;
- the extent to which we acquire or in-license other drugs and technologies;
- the costs of future commercialization activities, including drug sales, marketing, manufacturing and distribution, for any of our drug candidates for which we receive marketing approval, to the extent that such sales, marketing, manufacturing and distribution are not the responsibility of any collaborator that we may have at such time;
- the amount of revenue, if any, received from commercial sales of our drug candidates, should any of our drug candidates receive marketing approval; and
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims.

Identifying potential drug candidates and conducting preclinical studies and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve drug sales. In addition, our drug candidates, if approved, may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of drugs that may not be commercially available for several years, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all. In addition, we may seek additional capital due to favorable market conditions or strategic considerations, even if we believe we have sufficient funds for our current or future operating plans.

Contractual Obligations

There have been no material changes to our contractual obligations described in Management s Discussion and Analysis of Financial Condition and Results of Operations in the 2017 Form 10-K.

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, restricted cash and investments of $141.5 million as of March 31, 2018. 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 all of our investments are in short-term securities. Due to the short-term duration of our investment portfolio and the low risk profile of our investments, an immediate 10% change in interest rates would not have a material effect on the fair market value of our investment portfolio.

We do not believe our cash, cash equivalents, restricted cash and investments have significant risk of default or illiquidity. While we believe our cash, cash equivalents and investments do not contain excessive risk, we cannot provide absolute assurance that in the future our investments will not be subject to adverse changes in securities at one or more financial institutions that are in excess of federally insured limits. Give the potential instability of financial institutions, we cannot provide assurance that we will not experience losses on these deposits.

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We are also exposed to market risk related to change in foreign currency exchange rates. We contract with contract research organizations and contract manufacturing organizations that are located in Canada and Europe, which are denominated in foreign currencies. We also contract with a number of clinical trial sites outside the United States, and our budgets for those studies are frequently denominated in foreign currencies. We are subject to fluctuations in foreign currency rates in connection with these agreements. We do not currently hedge our foreign currency exchange rate risk.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer (principal executive officer) and Executive Vice President, Chief Financial Officer and Treasurer (principal financial officer), evaluated the effectiveness of our disclosure controls and procedures as of March 31, 2018. The term disclosure controls and procedures, as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended (the Exchange Act), means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC s rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the Company s management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of March 31, 2018, our Chief Executive Officer and our Executive Vice President, Chief Financial Officer and Treasurer 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

No change in our internal control over financial reporting occurred during the fiscal quarter ended March 31, 2018 that has materially affected, or is reasonably likely to materially affect, the Company s internal control over financial reporting.

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PART II OTHER INFORMATION

Item 1A. Risk Factors.

Careful consideration should be given to the following risk factors, in addition to the other information set forth in this Quarterly Report on Form 10-Q and in other documents that we file with the SEC, in evaluating the Company and our business. Investing in our common stock involves a high degree of risk. If any of the following risks and uncertainties actually occurs, our business, prospects, financial condition and results of operations could be materially and adversely affected. The risks described below are not intended to be exhaustive and are not the only risks facing the Company. New risk factors can emerge from time to time, and it is not possible to predict the impact that any factor or combination of factors may have on our business, prospects, financial condition and results of operations.

Risks Related to the Discovery, Development and Commercialization of Our Drug Candidates

We depend heavily on the success of our lead drug candidate selinexor (KPT-330), which is currently in clinical trials. Our clinical trials of selinexor may not be successful. If we are unable to commercialize selinexor or 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 lead drug candidate, selinexor. Our ability to generate revenues from the sale of drugs that treat cancer and other diseases in humans, which may not occur for several years, if ever, will depend heavily on the successful development, regulatory approval and eventual commercialization of selinexor.

We cannot commercialize drug candidates in the United States without first obtaining regulatory approval for the drug from the U.S. Food and Drug Administration, or FDA; similarly, we cannot commercialize drug candidates outside of the United States without obtaining regulatory approval from similar regulatory authorities outside of the United States. Even if selinexor or another drug candidate were to successfully obtain approval from the FDA and non-U.S. regulatory authorities, any approval might contain significant limitations related to use restrictions for specified age groups, warnings, precautions or contraindications, or may be subject to burdensome post-approval study or risk management requirements. If we are unable to obtain regulatory approval for selinexor in one or more jurisdictions, or any approval contains significant limitations, we may not be able to obtain sufficient funding or generate sufficient revenue to continue the development, marketing and/or commercialization of selinexor or any other drug candidate that we may discover, in-license, develop or acquire in the future. Furthermore, even if we obtain regulatory approval for selinexor, we will still need to develop a commercial organization, or collaborate with third parties, for the commercialization of selinexor, establish commercially viable pricing and obtain approval for adequate reimbursement from third-party and government payors. If we or our commercialization collaborators are unable to successfully commercialize selinexor, we may not be able to generate sufficient revenues to continue our business.

The results of previous clinical trials may not be predictive of future results, and the results of our current and planned clinical trials may not satisfy the requirements of the FDA or non-U.S. regulatory authorities.

We currently have no drugs approved for sale and we cannot guarantee that we will ever have marketable drugs. Clinical failure can occur at any stage of clinical development. Clinical trials may produce negative or inconclusive results, and we or any collaborators may decide, or regulators may require us, to conduct additional clinical trials or preclinical studies. We will be required to demonstrate with substantial evidence through well-controlled clinical trials that our drug candidates are safe and effective for use in a diverse population before we can seek regulatory approvals

for their commercial sale. Success in early-stage clinical trials does not mean that future larger registration clinical trials will be successful because drug candidates in later-stage clinical trials may fail to demonstrate sufficient safety and efficacy to the satisfaction of the FDA and non-U.S. regulatory authorities despite having progressed through early-stage clinical trials. Drug candidates that have shown promising results in early-stage clinical trials may still suffer significant setbacks in subsequent registration clinical trials. Additionally, the outcome of preclinical studies and early-stage clinical trials may not be predictive of the success of later-stage clinical trials, and interim results of a clinical trial are not necessarily indicative of final results. For example, we recently released top-line results from the expansion of our Selinexor Treatment of Refractory Myeloma (STORM) study. While we believe the results we have observed to date are positive, there can be no assurance that results that we believe to be positive will be viewed similarly by regulatory authorities or as sufficient to support a request for registration.

In addition, the design of a clinical trial can determine whether its results will support approval of a drug, and flaws in the design of a clinical trial may not become apparent until the clinical trial is well advanced. We may be unable to design and conduct a clinical trial to support regulatory approval. Further, if our drug candidates are found to be unsafe or lack efficacy, we will not be able to obtain regulatory approval for them and our business would be harmed. A number of companies in the pharmaceutical industry, including those with greater resources and experience than us, have suffered significant setbacks in advanced clinical trials, even after obtaining promising results in earlier clinical trials.

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In some instances, there can be significant variability in safety and/or efficacy results between different trials of the same drug 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. We do not know whether any Phase 2, Phase 3 or other clinical trials we may conduct will demonstrate consistent or adequate efficacy and safety sufficient to obtain regulatory approval to market our drug candidates.

Further, our drug candidates may not be approved even if they achieve their primary endpoints in Phase 3 clinical trials or other registration trials. The FDA or non-U.S. regulatory authorities may disagree with our trial design and our interpretation of data from preclinical studies and clinical trials. In addition, any of these regulatory authorities may change requirements for the approval of a drug candidate even after providing a positive opinion on, or otherwise reviewing and providing comments or advice on, a protocol for a clinical trial that has the potential to result in approval by the FDA or another regulatory authority. In addition, any of these regulatory authorities may also approve a drug candidate for fewer or more limited indications than we request or may grant approval contingent on the performance of costly post-marketing clinical trials. Furthermore, the FDA or other non-U.S. regulatory authorities may not approve the labeling claims that we believe would be necessary or desirable for the successful commercialization of our drug candidates.

To date, we have had several discussions with the FDA and non-U.S. regulatory authorities regarding the design of our later phase clinical trials for selinexor, including the BOSTON, STORM, SADAL and SEAL studies currently underway. We plan to seek regulatory approvals of selinexor in North America and Europe in each indication with respect to which such later phase clinical trial is being conducted and with respect to which we receive positive results that may support full or accelerated approval, as the case may be. We or our current or future partners may also seek such approvals in other geographies. We cannot be certain that we will commence additional later phase trials or complete ongoing later phase trials as anticipated. Before obtaining regulatory approvals for the commercial sale of any drug candidate for a target indication, we must demonstrate with substantial evidence gathered in preclinical studies and well-controlled clinical studies, and, with respect to approval in the United States, to the satisfaction of the FDA, that the drug candidate is safe and effective for use for that target indication. There is no assurance that the FDA or non-U.S. regulatory authorities would consider our current and planned later phase clinical trials to be sufficient to serve as the basis for filing for approval or to gain approval of selinexor for any indication. The FDA and non-U.S. regulatory authorities retain broad discretion in evaluating the results of our clinical trials and in determining whether the results demonstrate that selinexor is safe and effective. If we are required to conduct additional clinical trials of selinexor prior to approval, including additional earlier phase clinical trials that may be required prior to commencing any later phase clinical trials, or additional clinical trials following completion of our current and planned later phase clinical trials, we will need substantial additional funds, and there is no assurance that the results of any such additional clinical trials will be sufficient for approval.

The results to date in preclinical and early clinical studies conducted by us or our academic collaborators and in Phase 1 and Phase 2 clinical trials that we are currently conducting include the response of tumors to selinexor. We expect that in any later phase clinical trial where patients are randomized to receive either selinexor on the one hand, or standard of care, supportive care or placebo on the other hand, the primary endpoint will be either progression free survival, meaning the length of time on treatment until objective tumor progression, or overall survival, while the primary endpoint in any later phase clinical trial that is not similarly randomized may be different. For example, the primary endpoint of our Phase 2/3 SEAL study, the clinical trial of selinexor in patients with dedifferentiated liposarcoma, and a primary endpoint of our Phase 3 BOSTON study, the clinical trial of selinexor in combination with Velcade (bortezomib) and dexamethasone in patients with multiple myeloma, is progression free survival. We are in the early stages of collecting clinical data in humans relating to the impact of selinexor on overall survival and comparative clinical data between selinexor and supportive care. If selinexor does not demonstrate an overall survival

benefit, it will likely not be approved. In some instances, the FDA and other regulatory bodies have accepted overall response rate as a surrogate for a clinical benefit, and have granted regulatory approvals based on this or other surrogate endpoints. Overall response rate is defined as the portion of patients with tumor size reduction of a predefined amount for a minimum time period. For some types of cancer, we may use overall response rate as a primary endpoint, as we are doing in our SADAL study and our STORM study. These clinical trials will not be randomized against control arms and the primary endpoints of these trials are overall response rate. If selinexor does not demonstrate sufficient overall response rates in these indications, or any other indication for which a clinical trial has overall response rate as a primary endpoint, or if the FDA or non-U.S. regulatory authorities do not deem overall response rate a sufficient endpoint, or deem a positive overall response rate to be insufficient, it will likely not be approved for that indication based on the applicable study.

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We are early in our development efforts with a limited number of drug candidates in human clinical development. If we are unable to successfully develop and commercialize our drug candidates or experience significant delays in doing so, our business will be materially harmed.

We are early in our development efforts and have four drug candidates, selinexor, verdinexor, eltanexor and KPT-9274, in clinical development for treatment of human diseases. The success of these and any of our other drug candidates will depend on several factors, including the following:

- successful completion of preclinical studies;
- acceptance by the FDA of investigational new drug applications, or INDs, for our drug candidates prior to commencing clinical studies;
- successful enrollment in, and completion of, clinical trials, including demonstration of a favorable risk-benefit ratio;
- receipt of marketing approvals from applicable regulatory authorities;
- establishing commercial manufacturing capabilities or making arrangements with third-party manufacturers;
- obtaining and maintaining patent and trade secret protection and regulatory exclusivity for our drug candidates;
- establishing sales, marketing, manufacturing and distribution capabilities to commercialize any drugs for which we may obtain marketing approval;
- launching commercial sales of the drugs, if and when approved, whether alone or in collaboration with others;
- acceptance of the drugs, if and when approved, by patients, the medical community and third-party payors;
- effectively competing with other therapies;
- obtaining and maintaining coverage and adequate reimbursement by third-party payors, including government payors, for any approved drugs;

maintaining an acceptable safety profile of the drugs following approval;

enforcing and defending intellectual property rights and claims; and

maintaining and growing an organization of scientists and business people, including collaborators, who can develop and commercialize our drug candidates.

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 drug candidates, which would materially harm our business.

Our approach to the discovery and development of drug candidates that target Exportin 1, or XPO1, is unproven, and we do not know whether we will be able to develop any drugs of commercial value. If selinexor is unsuccessful in proving that drug candidates targeting XPO1 have commercial value or experiences significant delays in doing so, our business may be materially harmed.

Our SINE compounds inhibit the nuclear export protein XPO1. We believe that no currently approved cancer treatments are selectively targeting the restoration and increase in the levels of multiple tumor suppressor proteins in the nucleus. Despite promising results to date in preclinical studies of selinexor that we have conducted and in Phase 1 and Phase 2 clinical trials of selinexor conducted by us or our academic collaborators, we may not succeed in demonstrating safety and efficacy of SINE compounds in our current and future human clinical trials. Any drug candidates that we develop may not effectively prevent the exportation of tumor suppressor and/or growth regulatory proteins from the nucleus in humans with a particular form of cancer. If selinexor is unsuccessful in supporting the hypothesis that drug candidates targeting the regulation of intracellular transport of XPO1 have commercial value or experiences significant delays in doing so, our business may be materially harmed and we may not be able to generate sufficient revenues to continue our business.

We may not be successful in our efforts to identify or discover additional potential drug candidates.

Part of our strategy involves identifying and developing drug candidates to build a pipeline of novel drug candidates. Our drug discovery efforts may not be successful in identifying compounds that are useful in treating cancer or other diseases. Our research programs may initially show promise in identifying potential drug candidates, yet fail to yield drug candidates for clinical development for a number of reasons, including:

the research methodology used may not be successful in identifying potential drug candidates;

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potential drug candidates may, on further study, be shown to have harmful side effects or other characteristics that indicate that they are unlikely to be drugs that will receive marketing approval and/or achieve market acceptance; or

potential drug candidates may not be effective in treating their targeted diseases.

Research programs to identify new drug candidates require substantial technical, financial and human resources. We may choose to focus our efforts and resources on a potential drug candidate that ultimately proves to be unsuccessful.

If we are unable to identify suitable compounds for preclinical and clinical development, we will not be able to obtain revenues from sale of drugs in future periods, which likely would result in significant harm to our financial position and adversely impact our stock price.

Clinical drug development is a lengthy and expensive process, with an uncertain outcome. If clinical trials of our drug 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 drug candidates.

Before obtaining marketing approval from regulatory authorities for the sale of our drug candidates, we must complete preclinical development and then conduct extensive clinical trials to demonstrate the safety and efficacy of our drug 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 studies and early-stage 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, certain data from our Phase 1 and Phase 2 clinical trials of selinexor to date are based on unaudited data provided by our clinical trial investigators. An audit of this data may change the conclusions drawn from this unaudited data provided by our clinical trial investigators indicating less promising results than we currently anticipate. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that believed their drug candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their drugs.

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 drug candidates, including:

regulatory authorities 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;

feedback from regulatory authorities that requires us to modify the design of our clinical trials;

we may have delays in reaching or fail to reach agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites or contract research organizations;

clinical trials of our drug candidates may produce negative or inconclusive results, and we may decide, or regulatory authorities may require us, to conduct additional clinical trials, suspend ongoing clinical trials or abandon drug development programs;

the number of patients required for clinical trials of our drug candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate or 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;

we or our investigators might have to suspend or terminate clinical trials of our drug candidates for various reasons, including non-compliance with regulatory requirements, a finding that our drug candidates have undesirable side effects or other unexpected characteristics, or a finding that the participants are being exposed to unacceptable health risks;

the cost of clinical trials of our drug candidates may be greater than we anticipate;

the supply or quality of our drug candidates or other materials necessary to conduct clinical trials of our drug candidates may be insufficient or inadequate;

regulators may revise the requirements for approving our drug candidates, or such requirements may not be as we anticipate; and

any partners and collaborators that help conduct clinical trials may face any of the above issues, and may conduct clinical trials in ways they view as advantageous to them but that are suboptimal for us.

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

- be delayed in obtaining marketing approval for our drug candidates;
- not obtain marketing approval at all;
- obtain marketing approval in some countries and not in others;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings, including boxed warnings;
- be subject to additional post-marketing testing requirements; or
- have the drug removed from the market after obtaining marketing approval.

Our drug development costs will also increase if we experience delays in testing or marketing approvals. We do not know whether clinical trials will begin as planned, will need to be restructured or will be completed on schedule, or at all. Significant clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our drug candidates, allow our competitors to bring drugs to market before we do or impair our ability to successfully commercialize our drug candidates, which would harm our business and results of operations.

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

We may not be able to initiate or continue clinical trials for our drug candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA or similar regulatory authorities outside of the United States. In addition, some of our competitors may have ongoing clinical trials for drug candidates that treat the same indications as our drug candidates, and patients who would otherwise be eligible for our clinical trials may instead enroll in clinical trials of our competitors drug candidates.

Patient enrollment is affected by other factors, including:

- severity of the disease under investigation;

availability and efficacy of approved drugs for the disease under investigation;

patient eligibility criteria for the study in question;

competing drugs in clinical development;

perceived risks and benefits of the drug candidate under study;

restrictions on our ability to conduct clinical trials, including full or partial clinical holds on ongoing or planned trials;

efforts to facilitate timely enrollment in clinical trials;

patient referral practices of physicians;

the ability to monitor patients adequately during and after treatment; and

proximity and availability of clinical trial sites for prospective patients.

In addition, patient enrollment may be affected by future regulatory actions, such as Form 483 observations or the partial clinical hold we were subject to previously. In February 2017, following the conclusion of a joint inspection conducted by the FDA and Danish Medicines Agency at our corporate headquarters, the FDA issued a Form 483 noting certain deficiencies in procedures and documentation that were identified in our selinexor development program. We implemented corrective actions, preventative actions and other initiatives directed at resolving the deficiencies identified in the Form 483 observations and provided the FDA with our responses to the Form 483 observations in February 2017.

In addition, in March 2017, the FDA notified us that it had placed the clinical trials under our IND for selinexor on partial clinical hold, which is an order by the FDA to delay or suspend part of a sponsor s clinical work requested under its IND as well as investigator-sponsored trials. The partial clinical hold was due to incomplete information in the existing version of the investigator s brochure, including an incomplete list of serious adverse events associated with selinexor, and not as a result of any new information

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regarding the safety profile of selinexor. The partial clinical holds on the clinical trials of selinexor were lifted by the FDA Division of Hematology Products (effective March 30, 2017), Division of Oncology Products 1 (effective April 5, 2017) and Division of Oncology Products 2 (effective March 31, 2017). However, if in the future we are delayed in addressing, or unable to address, any concerns of the FDA or other regulators, we could be delayed or prevented from enrolling patients in our clinical trials.

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

If serious adverse or unacceptable side effects are identified during the development of our drug candidates or we observe limited efficacy of our drug candidates, we may need to abandon or limit the development of one or more of our drug candidates.

Four of our drug candidates are in clinical development for treatment of human diseases. Their risk of failure is high. It is impossible to predict when or if any of our drug candidates will prove effective or safe in humans or will receive marketing approval. If our drug candidates are associated with undesirable side effects or have characteristics that are unexpected, we may need to abandon their development or limit development to certain uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective. For example, we have modified our informed consent form and advised patients already enrolled in our clinical trials of the potential for worsening of pre-existing cataracts as a result of treatment with selinexor. Also, even though selinexor has generally been well-tolerated by patients in our clinical trials to date, in some cases there were adverse events, some of which were serious. The most common drug-related adverse events, or AEs, were gastrointestinal, such as nausea, anorexia, diarrhea and vomiting, and fatigue. These side effects were generally mild or moderate in severity. The most common AEs that were Grade 3 or Grade 4, meaning they were more than mild or moderate in severity, were thrombocytopenia, or low count of platelets in the blood, and neutropenia, or low neutrophil counts. A small percentage of patients have withdrawn from our clinical trials as a result of AEs. A small percentage of patients across our clinical trials have experienced serious adverse events, or SAEs, deemed by us and the clinical investigator to be related to selinexor. SAEs generally refer to AEs that result in death, are life threatening, require hospitalization or prolonging of hospitalization, or cause a significant and permanent disruption of normal life functions, congenital anomalies or birth defects, or require intervention to prevent such an outcome.

As a result of these AEs or further safety or toxicity issues that we may experience in our clinical trials in the future, we may not receive approval to market any drug candidates, which could prevent us from ever generating revenue from the sale of drugs or achieving profitability. Results of our trials could reveal an unacceptably high severity and prevalence of side effects. In such an event, our trials could be suspended or terminated and the FDA or comparable foreign regulatory authorities could order us to cease further development of or deny approval of our drug candidates for any or all targeted indications. Many compounds that initially showed promise in early-stage trials for treating cancer or other diseases have later been found to cause side effects that prevented further development of the compound.

The FDA or non-U.S. regulatory authorities may disagree with our and/or our clinical trial investigators interpretation of data from clinical trials in determining if serious adverse or unacceptable side effects are drug-related.

We, and our clinical trial investigators, currently determine if serious adverse or unacceptable side effects are drug-related. The FDA or non-U.S. regulatory authorities may disagree with our or our clinical trial investigators interpretation of data from clinical trials and the conclusion by us or our clinical trial investigators that a serious adverse effect or unacceptable side effect was not drug-related. The FDA or non-U.S. regulatory authorities may require more information, including additional preclinical or clinical data to support approval, which may cause us to incur additional expenses, delay or prevent the approval of one of our drug candidates, and/or delay or cause us to change our commercialization plans, or we may decide to abandon the development or commercialization of the drug candidate altogether.

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

Because we have limited financial and managerial resources, we focus on research programs and drug candidates that we identify for specific indications. As a result, we may forego or delay pursuit of opportunities with other drug candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial drugs or profitable market opportunities. Our spending on current and future research and development programs and drug candidates for specific indications may not yield any commercially-viable drugs. If we do not accurately evaluate the commercial potential or target market for a particular drug candidate, we may relinquish valuable rights to that drug candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such drug candidate.

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Even if any of our drug candidates receives marketing approval, such drug may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success.

If any of our drug candidates receives marketing approva