
**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, 2014

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
(State or other jurisdiction of
incorporation or organization)

2 Mercer Road
Natick, MA
(Address of principal executive offices)

26-3931704
(I.R.S. Employer
Identification Number)

01760
(Zip Code)

(508) 975-4820
(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, or a smaller reporting company. See the definitions of "large accelerated filer," "accelerated filer" and "smaller reporting company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer
Non-accelerated filer
(Do not check if a smaller reporting company)

Accelerated filer
Smaller reporting company

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 April 30, 2014 there were 29,762,499 shares of Common Stock, \$0.0001 par value per share, outstanding.

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FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this Quarterly Report on Form 10-Q, including statements regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management, are forward looking statements. The words “anticipate,” “believe,” “estimate,” “expect,” “intend,” “may,” “plan,” “predict,” “project,” “target,” “potential,” “will,” “would,” “could,” “should,” “continue” and similar expressions are intended to identify forward-looking statements, although not all forward- looking statements contain these identifying words.

Forward-looking statements are not guarantees of future performance and our actual results could differ materially from the 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, 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 protect and maintain our intellectual property and the ability of our licensors to obtain and maintain patent protection for the technology or products that we license from them, 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 our Annual Report on Form 10-K and other filings with the Securities and Exchange Commission, or 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.

PART I—FINANCIAL INFORMATION

Item 1. Condensed Consolidated Financial Statements (Unaudited).

Karyopharm Therapeutics Inc.

(A development stage company)

CONDENSED CONSOLIDATED BALANCE SHEETS

(unaudited)

(in thousands, except share and per share amounts)

	March 31, 2014	December 31, 2013
Assets		
Current assets:		
Cash and cash equivalents	\$ 144,893	\$ 155,974
Prepaid expenses and other current assets	2,673	1,982
Total current assets	147,566	157,956
Property and equipment, net	322	240
Other assets	997	30
Restricted cash	400	—
Total assets	<u>\$ 149,285</u>	<u>\$ 158,226</u>
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	\$ 3,273	\$ 1,740
Accrued expenses	1,550	1,168
Deferred revenue	57	79
Other short-term liabilities	135	305
Total current liabilities	5,015	3,292
Other long-term liabilities	170	—
Stockholders' equity		
Convertible preferred stock, \$0.0001 par value; 5,000,000 shares authorized; none issued and outstanding	—	—
Common stock, \$0.0001 par value; 100,000,000 shares authorized; 29,618,879 and 29,587,258 shares issued and outstanding at March 31, 2014 and December 31, 2013, respectively	3	3
Additional paid-in capital	220,360	217,500
Deficit accumulated during the development stage	(76,263)	(62,569)
Total stockholders' equity	144,100	154,934
Total liabilities and stockholders' equity	<u>\$ 149,285</u>	<u>\$ 158,226</u>

See accompanying notes to condensed consolidated financial statements.

Karyopharm Therapeutics Inc.

(A development stage company)

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(unaudited)

(in thousands, except share and per share amounts)

	Three months ended, March 31,		Period from December 22, 2008 (Inception) to March 31, 2014
	2014	2013	2014
Contract and grant revenue	\$ 171	\$ 233	\$ 1,437
Operating expenses:			
Research and development	10,979	4,965	63,814
General and administrative	2,904	879	13,721
Total operating expenses	13,883	5,844	77,535
Loss from operations	(13,712)	(5,611)	(76,098)
Interest income (expense), net	18	—	(165)
Net loss	\$ (13,694)	\$ (5,611)	\$ (76,263)
Net loss per share applicable to common stockholders—basic and diluted	\$ (0.46)	\$ (2.52)	\$ (24.70)
Weighted-average number of common shares outstanding used in net loss per share applicable to common stockholders—basic and diluted	29,606,683	2,225,596	3,087,622
Comprehensive loss	\$ (13,694)	\$ (5,611)	\$ (76,263)

See accompanying notes to condensed consolidated financial statements.

Karyopharm Therapeutics Inc.

(A development stage company)

CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS

(unaudited)

(in thousands)

	Three months ended March 31,		Period from December 22, 2008 (Inception) to March 31, 2014
	2014	2013	
Operating activities			
Net loss	\$ (13,694)	\$ (5,611)	\$ (76,263)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation and amortization	43	34	393
Stock-based compensation expense	2,846	204	7,354
Noncash consulting expense	—	—	788
Noncash interest expense	—	—	188
Changes in operating assets and liabilities:			
Prepaid expenses and other current assets	(691)	(120)	(2,673)
Other non-current assets	(967)	—	(997)
Accounts payable	1,533	376	3,273
Accrued expenses and other liabilities	383	(7)	1,553
Deferred revenue	(22)	(33)	57
Net cash used in operating activities	(10,569)	(5,157)	(66,327)
Investing activities			
Purchases of property and equipment	(125)	—	(715)
Increase in restricted cash	(400)	—	(400)
Net cash used in investing activities	(525)	—	(1,115)
Financing activities			
Net proceeds from issuance of common stock	—	—	113,177
Proceeds from the exercise of stock options	13	30	354
Proceeds from the issuance of convertible notes	—	—	250
Proceeds from the issuance of preferred stock subscription	—	3,000	—
Principal payments of convertible notes	—	—	(200)
Proceeds from sale of convertible preferred stock, net of issuance costs	—	2,500	98,754
Net cash provided by financing activities	13	5,530	212,335
Net increase (decrease) in cash and cash equivalents	(11,081)	373	144,893
Cash and cash equivalents at beginning of period	155,974	391	—
Cash and cash equivalents at end of period	\$ 144,893	\$ 764	\$ 144,893
Supplemental disclosure of non-cash financing activity			
Issuance of shares related to preferred stock subscription	\$ —	\$ —	\$ 13,980
Conversion of convertible preferred stock upon initial public offering	\$ —	\$ —	\$ 99,691
Conversion of notes payable to preferred stock	\$ —	\$ —	\$ 750
Issuance of convertible notes in satisfaction of accrued expenses	\$ —	\$ —	\$ 700

See accompanying notes to condensed consolidated financial statements.

Karyopharm Therapeutics Inc.

(A development stage company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

1. Summary of Significant Accounting Policies

Basis of Presentation

The accompanying unaudited condensed consolidated financial statements of Karyopharm Therapeutics, Inc (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, 2014 are not necessarily indicative of the results that may be expected for any other interim period or for the fiscal year ending December 31, 2014. 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, 2013 as filed with the Securities and Exchange Commission (“SEC”) on March 21, 2014.

Basis of Consolidation

The consolidated financial statements at March 31, 2014 include the accounts of Karyopharm Therapeutics Inc. (a Delaware corporation) and the accounts of Karyopharm Securities Corp (“KPSC”, a wholly-owned Massachusetts corporation), incorporated in December 2013. At December 31, 2013, the consolidated financial statements also included the accounts of NPM Pharma Inc. (“NPM”, a wholly-owned Canadian corporation). As of March 31, 2014, NPM’s assets and liabilities were transferred to Karyopharm Therapeutics Inc. Following the transfer, NPM was dissolved. The dissolution of NPM had no effect on the consolidated financial statements.

Subsequent Events

In preparing the financial statements included in this Form 10-Q, the Company has evaluated all subsequent events that occurred after March 31, 2014 through the date of the filing of this Form 10-Q. The Company did not have any material recognizable or unrecognizable subsequent events during this period.

2. Fair Value of Financial Instruments

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 is now established that 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

The following table presents information about the Company’s financial assets that have been measured at fair value at March 31, 2014 and indicates the fair value hierarchy of the valuation inputs utilized to determine such fair value (in thousands):

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Description	Total	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
Financial assets				
Money Market Funds, included in cash equivalents	\$ 144,775	\$ 144,775	\$ —	\$ —

The following table presents information about the Company's financial assets that have been measured at fair value at December 31, 2013 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				
Money Market Funds, included in cash equivalents	\$ 155,765	\$ 155,765	\$ —	\$ —

The Company's cash equivalents are comprised of money market funds. The Company measures these investments at fair value. The fair value of cash equivalents is determined based on "Level 1" inputs.

3. Property and Equipment, net

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

	Estimated Useful Life Years	March 31, 2014	December 31, 2013
Laboratory equipment	4	\$ 359	\$ 328
Furniture and fixtures	5	103	98
Office and computer equipment	3	85	85
Leasehold improvements	Lease Term	79	79
Construction in process	n/a	89	—
		715	590
Less accumulated depreciation and amortization		(393)	(350)
		\$ 322	\$ 240

4. Accrued Expenses

Accrued expenses consist of the following (in thousands):

	March 31, 2014	December 31, 2013
Professional fees	\$ 469	\$ 215
Payroll and employee-related costs	415	100
Research and development costs	314	698
Other	352	155
	\$ 1,550	\$ 1,168

5. Net Loss per Share

Basic and diluted net loss per common share is calculated by dividing net loss applicable to common stockholders by the weighted-average number of common shares outstanding during the period, without consideration for common stock equivalents. The Company's potentially dilutive shares, which include convertible preferred stock, special participation stock, outstanding stock options and unvested restricted stock 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,		Period from December 22, 2008 (inception) to March 31, 2014
	2014	2013	2014
Convertible preferred stock	—	20,937,500	—
Special participation stock	—	10,000	—
Outstanding stock options	2,505,749	607,241	2,505,749
Unvested restricted stock	143,620	477,909	143,620

6. Stock-based Compensation

During 2010, the Company established the 2010 stock incentive plan (the "2010 plan"). In October 2013, the Company adopted the 2013 Stock Incentive Plan (the "2013 Plan"). The 2013 Plan became effective upon the closing of the Company's initial public offering ("IPO") in November 2013. The 2013 Plan provides for the grant of incentive stock options, nonstatutory stock options, stock appreciation rights, restricted stock awards, restricted stock units and other stock-based awards. Upon effectiveness of the IPO, the number of shares of common stock that were reserved under the 2013 Plan was the sum of 969,696 shares plus 198,372, the number of shares available under the 2010 Plan. The number of shares reserved under the 2013 Plan is increased by the number of shares of common stock (up to a maximum of 2,126,377 shares) subject to outstanding awards under the 2010 Plan that expire, terminate or are otherwise surrendered, cancelled, forfeited or repurchased by the Company. The 2013 Plan includes an "evergreen provision" that allows for an annual increase in the number of shares of common stock available for issuance under the 2013 Plan. The annual increase will be added on the first day of each year beginning in 2014 and each subsequent anniversary until the expiration of the 2013 Plan, equal to the lowest of: (i) 1,939,393 shares of common stock, (ii) 4.0% of the number of shares of common stock outstanding and (iii) an amount determined by the board of directors. On January 1, 2014, the shares available under the 2013 Plan increased by 1,190,149 shares of common stock. No additional awards may be granted under the 2010 plan.

Restricted stock

A summary of the Company's unvested restricted stock as of March 31, 2014 and changes during the three months ended March 31, 2014 is as follows:

	Shares	Weighted- average purchase price per share
Unvested at December 31, 2013	9,943	\$ 0.26
Vested	(1,411)	0.26
Unvested at March 31, 2014(1)	8,532	\$ 0.26

(1) Excludes 135,088 shares of unvested restricted stock remaining from the early exercise of stock options.

As of March 31, 2014, there was \$261,000 of total unrecognized stock-based compensation expense related to unvested restricted stock. The expense is expected to be recognized over a weighted average period 1.4 years.

Stock options

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

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	Shares	Weighted-average price per share	Weighted-average remaining contractual term (years)	Aggregate intrinsic value (in thousands)
Outstanding at December 31, 2013	2,410,522	\$ 7.85	9.3	\$ 36,717
Granted	104,000	40.14		
Exercised	(8,773)	1.49		
Canceled	—	—		
Outstanding at March 31, 2014	<u>2,505,749</u>	<u>\$ 9.21</u>	<u>9.1</u>	<u>\$ 55,292</u>
Exercisable at March 31, 2014	<u>359,420</u>	<u>\$ 0.41</u>	<u>7.3</u>	<u>\$ 10,957</u>
Vested and expected to vest at March 31, 2014	<u>2,325,848</u>	<u>\$ 9.21</u>	<u>9.1</u>	<u>\$ 51,339</u>

As of March 31, 2014, there was \$22.0 million of total unrecognized stock-based compensation expense related to stock options. The expense is expected to be recognized over a weighted average period 3.5 years.

7. Commitments and Contingencies

In March 2014, the Company entered into an operating lease for approximately 29,933 square feet of office and research space. The Company intends to use the leased premises as its corporate headquarters and for research and development purposes. The lease term commences May 2014 and expires in October 2021. Pursuant to the lease agreement, the Company is obligated to make aggregate rent payments of \$5.6 million through October 2021. There are no scheduled rent payments due for the first 23 weeks of the lease term. Thereafter, the Company has agreed to pay an initial annual base rent of approximately \$506,000, which base rent rises periodically until it reaches approximately \$898,000. The Company has agreed to pay for pro rata increases in operating expenses and property taxes. The lease provides the Company with an allowance for improvements of \$1.0 million and an ability to finance up to \$449,000 at 8% annual interest, amortized over the term of the lease. The Company has provided a security deposit in the form of a letter of credit in the amount of \$400,000, which amount may be reduced to \$200,000 in January 2018.

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. The following discussion contains forward-looking statements that involve risks and uncertainties. Our actual results and the timing of certain events could differ materially from those anticipated in these forward-looking statements as a result of certain factors, including those discussed below and elsewhere in this quarterly report or in our annual report on Form 10-K.

OVERVIEW

We are a clinical-stage pharmaceutical company focused on the discovery and development of novel first-in-class drugs directed against nuclear transport targets for the treatment of cancer and other major diseases. Our scientific expertise is focused on the understanding of the regulation of intracellular transport between the nucleus and the cytoplasm. We have discovered and developed novel, small molecule, **Selective Inhibitors of Nuclear Export**, or **SINE**, compounds that inhibit the nuclear export protein XPO1. We have worldwide rights to these SINE compounds. Our lead drug candidate, Selinexor (KPT-330), is a first-in-class, oral SINE compound. Selinexor functions by binding with the nuclear export protein XPO1 (also called CRM1), leading to the accumulation of tumor suppressor proteins in the cell nucleus, which subsequently reinitiates and amplifies their tumor suppressor function. This is believed to lead to the selective induction of apoptosis in cancer cells, while largely sparing normal cells. To date, over 300 patients have been treated with Selinexor in Phase 1 and Phase 2 trials in advanced hematologic malignancies and solid tumors. Additional Phase 1 and Phase 2 studies are ongoing or currently planned and three registration-directed clinical trials in hematological indications are expected to begin enrollment during 2014.

We have devoted substantially all of our efforts to research and development. We expect that it will be several years, if ever, before we have a drug candidate ready for commercialization for the treatment of human disease. To date, we have financed our operations primarily with the net proceeds from the private placements of our preferred stock and the net proceeds from our IPO.

As of March 31, 2014, we had a deficit accumulated during the development stage of \$76.3 million. We had net losses of \$13.7 million and \$5.6 million for the three months ended March 31, 2014 and 2013, respectively. We have not generated any revenue to date from 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;
- identify additional drug candidates;
- initiate additional clinical trials for our drug candidates;
- seek marketing approvals for any of our drug candidates that successfully complete clinical trials;
- ultimately 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;
- hire additional clinical, quality control and scientific personnel;
- 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 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.

The critical accounting policies we identified in our most recent Annual Report on Form 10-K for the fiscal year ended December 31, 2013 related to accrued research and development expenses and stock-based compensation. There were no changes to these critical accounting policies in the quarter ended March 31, 2014. It is important that the discussion of our

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operating results that follows be read in conjunction with the critical accounting policies disclosed in our Annual Report on Form 10-K, as filed with the SEC on March 21, 2014.

RESULTS OF OPERATIONS

Comparison of the Three Months ended March 31, 2014 and March 31, 2013

	Three Months Ended March 31,	
	2014	2013
	(in thousands)	
Contract and grant revenue	\$ 171	\$ 233
Operating expenses:		
Research and development	10,979	4,965
General and administrative	2,904	879
Loss from operations	(13,712)	(5,611)
Interest income	18	—
Net loss	\$ (13,694)	\$ (5,611)

Contract and Grant Revenue. The Company recognizes revenue pursuant to sponsored research agreements. Contract and grant revenue for the three months ended March 31, 2014 (“the 2014 Quarter”) was \$171,000 compared to \$233,000 for the three months ended March 31, 2013 (“the 2013 Quarter”). The decrease in revenue was the result of recognizing smaller milestones associated with grants during the 2014 Quarter.

Research and Development Expense. Research and development expense increased by \$6.0 million to \$11.0 million in the 2014 Quarter from \$5.0 million in the 2013 Quarter. The \$6.0 million increase is primarily related to:

- an increase of \$1.5 million in consulting fees, including an increase of \$868,000 in stock-based compensation expense related to equity awards granted to consultants, primarily due to the higher fair value of our common stock,
- an increase of \$1.4 million in personnel costs, primarily due to increased headcount and an increase of \$681,000 in stock-based compensation expense related to equity awards granted to personnel, primarily related to the higher fair value of our common stock,
- an increase of \$1.3 million in clinical trial costs, including an increase of \$210,000 in the cost of finished drug product,
- an increase of \$961,000 in discovery work, including preclinical studies and screening, and
- an increase of \$719,000 in IND-enabling toxicology studies.

General and Administrative Expense. General and administrative expense increased by \$2.0 million to \$2.9 million for the 2014 Quarter from \$879,000 for the 2013 Quarter. The \$2.0 million increase is primarily related to:

- an increase of \$864,000 in personnel costs, primarily due to increased headcount and an increase of \$554,000 in stock-based compensation expense related to equity awards granted to personnel, primarily related to the higher fair value of our common stock.
- an increase of \$475,000 in consulting fees, primarily related to investor relations and business development, including an increase of \$540,000 in stock-based compensation expense related to equity awards granted to consultants, primarily due to the higher fair value of our common stock,
- an increase of \$395,000 in professional fees, primarily related to higher legal fees for protecting our intellectual property, corporate legal fees and audit fees,
- an increase of \$166,000 in insurance expense, primarily due to our becoming a publicly traded company, and
- an increase of \$114,000 in state tax expenses.

Interest income. Interest income increased to \$18,000 for the 2014 Quarter from none for the 2013 Quarter. This increase is due to a higher cash and cash equivalents balance during the 2014 Quarter compared to the 2013 Quarter.

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 and proceeds from our initial public offering. As of March 31, 2014, we had \$144.9 million in cash and cash equivalents. We primarily invest our cash and cash equivalents in a money market fund. We expect that our existing cash and cash equivalents will enable us to fund our operating expenses and capital expenditure requirements into early 2016.

Cash Flows

The following table provides information regarding our cash flows:

	Three Months Ended March 31,	
	2014	2013
	(in thousands)	
Net cash used in operating activities	\$ (10,569)	\$ (5,157)
Net cash used in investing activities	(525)	—
Net cash provided by financing activities	13	5,530
Net (decrease) increase in cash and cash equivalents	\$ (11,081)	\$ 373

Operating activities. The cash used in operating activities in all periods resulted primarily from our net losses adjusted for non-cash charges and changes in the components of working capital. The significant increase in cash used in operating activities for the 2014 Quarter compared to the 2013 Quarter is due to an increase in research and development expenses as we increased our research and development headcount and increased spending on external research and development costs.

Investing activities. The cash used in investing activities for the 2014 Quarter reflects an increase of \$400,000 in restricted cash related to a facility lease and the purchase of \$125,000 of property and equipment. There were no investing activities in the 2013 Quarter.

Financing activities. The cash provided by financing activities for the 2014 Quarter reflects the proceeds from the exercise of stock options. The cash provided by financing activities in the 2013 Quarter was primarily from the sale of preferred stock and issuance of preferred stock subscriptions.

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 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 existing cash and cash equivalents will enable us to fund our current operating plan and capital expenditure requirements into early 2016. Our future capital requirements will depend on many factors, including:

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

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- 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 we do not expect to be commercially available for many 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

As of March 31, 2014, we had the following contractual obligations:

Contractual Obligations	Payments Due by Period				
	Total	Less than 1 Year	2-3 Years	4-5 Years	More than 5 Years
	(in thousands)				
Operating lease obligations(1)	\$ 5,689	\$ 350	\$ 1,418	\$ 1,706	\$ 2,215
Purchase obligations(2)	—	—	—	—	—
Total contractual cash obligations	\$ 5,689	\$ 350	\$ 1,418	\$ 1,706	\$ 2,215

(1) Represents future minimum lease payments under our non-cancelable operating lease.

(2) We enter into agreements in the normal course of business with Contract Research Organizations (CROs) and Contract Manufacturing Organizations (CMOs) for clinical trials and clinical supply manufacturing and with vendors for preclinical research. We have not included these payments in the table of contractual obligations above since the contracts are cancelable at any time by us, generally upon 30 days prior written notice to the vendor.

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 Securities and Exchange Commission 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 and cash equivalents of \$144.9 million as of March 31, 2014, consisting of cash and U.S. Treasury money market funds. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because most of our investments are in short-term securities. Due to the short-term duration most 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 are also exposed to market risk related to change in foreign currency exchange rates. We contract with CROs and

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CMOs that are located in Canada and Europe, which are 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 and Senior Vice President, Finance and Administration, evaluated the effectiveness of our disclosure controls and procedures as of March 31, 2014. 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, 2014, our Chief Executive Officer and Senior Vice President, Finance and Administration 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 three months ended March 31, 2014 that has materially affected, or is reasonably likely to materially affect, the Company’s internal control over financial reporting.

PART II—OTHER INFORMATION

Item 1. Legal Proceedings.

None.

Item 1A. Risk Factors.

You should carefully review and consider the information regarding certain factors that could materially affect our business, financial condition or future results set forth under Item 1A. (Risk Factors) in our Annual Report on Form 10-K for the fiscal year ended December 31, 2013 filed with the SEC on March 21, 2014. There have been no material changes from the factors disclosed in our 2013 Annual Report on Form 10-K, although we may disclose changes to such factors or disclose additional factors from time to time in our future filings with the Securities and Exchange Commission.

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

RECENT SALES OF UNREGISTERED SECURITIES

None.

PURCHASE OF EQUITY SECURITIES

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

USE OF PROCEEDS FROM REGISTERED SECURITIES

On November 12, 2013, we issued and sold 6,800,000 shares of our common stock in the IPO at a public offering price of \$16.00 per share, for aggregate gross proceeds of \$108.8 million. On December 10, 2013, we issued and sold 1,020,000

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shares of our common stock pursuant to the underwriters' full exercise of their option to purchase additional shares in the IPO at \$16.00 per share for gross proceeds of \$16.3 million. All of the shares issued and sold in the IPO were registered under the Securities Act of 1933, as amended (the "Securities Act") pursuant to a Registration Statement on Form S-1 (File No. 333-191584), which was declared effective by the SEC on November 5, 2013, and a Registration Statement on Form S-1 (File No. 333-192110) filed pursuant to Rule 462(b) of the Securities Act. Merrill Lynch, Pierce, Fenner & Smith Incorporated and Leerink Swann LLC acted as joint-book-running managers of the offering and as representatives of the underwriters. JMP Securities LLC and Oppenheimer & Co. Inc. acted as co-managers for the offering. The offering commenced on November 5, 2013 and terminated upon sale of all of the shares offered.

The net offering proceeds to us, after deducting underwriting discounts of \$8.8 million and offering expenses payable by us totaling \$3.2 million, were approximately \$113.2 million. No offering expenses were paid directly or indirectly to any of our directors or officers (or their associates) or persons owning 10.0% or more of any class of our equity securities or to any other affiliates.

As of March 31, 2014, we have used approximately \$16.9 million of such net offering proceeds to fund the continued clinical development of our lead drug candidate, Selinexor (KPT-330), the preclinical development of our drug candidates for anti-inflammatory, viral and wound-healing indications, the discovery, research, preclinical development and clinical trials of additional drug candidates and for working capital and other general corporate purposes. We are holding a significant portion of the balance of the net proceeds from the offering in interest-bearing money market accounts and prime money market funds. There has been no material change in our planned use of the balance of the net proceeds from the offering described in the prospectus filed by us with the SEC pursuant to Rule 424(b)(4) on November 7, 2013.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

None.

Item 5. Other Information.

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

On May 7, 2014, Karyopharm Therapeutics Inc. announced its financial results for the quarter ended March 31, 2014 and commented on certain corporate accomplishments and plans. The full text of the press release issued in connection with the announcement is furnished as Exhibit 99.1 hereto.

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

Item 6. Exhibits.

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

EXHIBIT INDEX

Exhibit Number	Description of Exhibit	Form	Incorporated by Reference			Filed Herewith
			File Number	Date of Filing	Exhibit Number	
10.1	Offer Letter from the Registrant to Christopher Primiano dated March 3, 2014	10-K	001-36167	03/21/14	10.13	
10.2	Office Lease Agreement between NS Wells Acquisition LLC and the Registrant dated March 27, 2014.	8-K	011-36167	04/01/14	10.1	
31.1	Certification of principal executive officer pursuant to Rule 13a-14(a)/15d-14(a) of the Securities Exchange Act of 1934, as amended.					X
31.2	Certification of principal financial officer pursuant to Rule 13a-14(a)/15d-14(a) of the Securities Exchange Act of 1934, as amended.					X
32.1	Certification of principal executive officer pursuant to 18 U.S.C. §1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
32.2	Certification of principal financial officer pursuant to 18 U.S.C. §1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
99.1	Press Release issued by Karyopharm Therapeutics Inc. on May 7, 2014.					X

CERTIFICATIONS

I, Michael Kauffman, M.D., Ph.D., certify that:

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

/s/ MICHAEL KAUFFMAN, M.D., PH.D.

Michael Kauffman, M.D., Ph.D.

Chief Executive Officer

Date: May 8, 2014

CERTIFICATIONS

I, Paul Brannelly, certify that:

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

/s/ PAUL BRANNELLY

Paul Brannelly

Senior Vice President, Finance and Administration

Date: May 8, 2014

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

In connection with the Quarterly Report on Form 10-Q of Karyopharm Therapeutics Inc. (the "Company") for the period ended March 31, 2014 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Michael Kauffman, M.D., Ph.D., Chief Executive Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to his knowledge:

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

/s/ MICHAEL KAUFFMAN, M.D., PH.D.

Michael Kauffman, M.D., Ph.D.

Chief Executive Officer

Date: May 8, 2014

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

In connection with the Quarterly Report on Form 10-Q of Karyopharm Therapeutics Inc. (the "Company") for the period ended March 31, 2014 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Paul Brannelly, Senior Vice President, Finance and Administration of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to his knowledge:

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

/s/ PAUL BRANNELLY

Paul Brannelly

Senior Vice President, Finance and Administration

Date: May 8, 2014



Karyopharm Therapeutics Reports First Quarter Financial Results for 2014

Natick, Mass. — May 7, 2014 — Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a clinical-stage pharmaceutical company focused on the discovery and development of novel first-in-class drugs directed against nuclear transport targets for the treatment of cancer and other major diseases, today reported financial results for the fiscal quarter ended March 31, 2014, and also provided highlights of its clinical development activity.

Dr. Michael Kauffman, CEO of Karyopharm, commented, “We believe Selinexor may be the first broad tumor suppressor protein activator with treatment potential in any cancer. Our clinical trials continue to accrue well, with a growing number of patients across a variety of heavily pretreated hematologic and solid tumor indications remaining on single agent oral Selinexor for prolonged periods. We have identified the recommended Phase 2/3 single agent dose for twice-weekly administration of Selinexor and are in the midst of initiating our first combination studies, along with randomized and single arm studies. With the proceeds from our IPO and prior private financings, we are able to move Selinexor forward on multiple fronts focusing on both registration-directed and exploratory Phase 2 trials as supported by the results of our ongoing Phase 1 studies.”

First Quarter 2014 Financial Results

Cash and cash equivalents as of March 31, 2014, totaled \$144.9 million compared with \$156.0 million as of December 31, 2013.

For the quarter ended March 31, 2014, research and development expense was \$11.0 million compared to \$5.0 million for the same period in the previous year. For the quarter ended March 31, 2014, general and administrative expense was \$2.9 million compared to \$879,000 for the same period in the previous year. The increase in expenses resulted primarily from the increase in expenses related to the continued clinical development of lead drug candidate Selinexor (KPT-330).

Karyopharm reported a net loss of \$13.7 million, or \$0.46 per share, for the quarter ended March 31, 2014, compared to a net loss of \$5.6 million, or \$2.52 per share, for the same period in the previous year. Net loss includes stock-based compensation expense of \$2.8 million and \$204,000 for the quarters ended March 31, 2014 and 2013, respectively.

Financial Guidance

Based on current operating plans, Karyopharm said it expects to have sufficient cash and cash equivalents to fund research and development programs and operations into early 2016. Karyopharm expects to end 2014 with approximately \$100 million in cash and cash equivalents.

Clinical Development Highlights

Over 300 patients have been treated with Karyopharm’s lead drug candidate, Selinexor (KPT-330), a first-in-class, oral Selective Inhibitor of Nuclear Export (SINE) compound, in Phase 1 and Phase 2 trials in advanced hematologic malignancies and solid tumors. Nine clinical trials to evaluate Selinexor have been initiated to date. The company expects to initiate up to five additional trials this year, along with numerous investigator-sponsored trials.

Ongoing Phase 1 Trials

Karyopharm continues to enroll patients in its three ongoing Phase 1 clinical trials for Selinexor (KPT-330) in advanced hematologic malignancies, solid tumors and sarcomas. In these studies, patients have progressive disease relapsed or refractory to essentially all available classes of therapeutic agents. The recommended Phase 2/3 dose for single agent oral Selinexor is 55-65 mg/m² twice weekly. This corresponds to 80-140 mg per dose. Once weekly dosing with Selinexor is currently being evaluated at doses of 80 mg/m² and higher. The use of appetite stimulants and anti-nausea agents in all patients who begin therapy with Selinexor has improved tolerability. A growing number of patients have been treated for over one year with no clinically significant cumulative toxicities and evidence of broad anti-cancer activity has been observed.

New Study Initiations

Karyopharm has announced the initiation of a number of additional studies for Selinexor.

- **First Combination Study.** In this combination study, patients with relapsed and/or refractory acute myeloid leukemia (AML) or newly diagnosed AML patients ≥ 60 years of age ineligible for intensive chemotherapy will receive decitabine intravenously on days 1-10 and Selinexor orally twice weekly beginning on day 11 of each 31-day cycle. The study is being conducted at The Ohio State University Comprehensive Cancer Center in up to 42 patients with a primary goal to determine the maximum tolerated dose and the recommended Phase 2/3 dose of this combination. The secondary goal of the study is to determine the response rates and duration of leukemia control.
- **First Study in Pediatric Patients.** This trial aims to determine the oral dosing, toxicity and preliminary clinical activity of Selinexor in pediatric leukemia patients. It will enroll up to 28 children with relapsed or refractory acute lymphoblastic leukemia (ALL) or AML. The study is being led by Dana-Farber/Boston Children's Cancer and Blood Disorders Center and is supported in part by a grant from the William Lawrence & Blanche Hughes Foundation.
- **Phase 2 Study in Patients with Advanced Gynecologic Malignancies (SIGN).** The primary goal of this Phase 2 study, known as the SIGN study, is to determine the disease control rate assessed according to RECIST criteria and will evaluate Selinexor in up to 63 patients with advanced gynecologic malignancies including cervical, ovarian and uterine carcinomas. The secondary goals of the study are to evaluate safety, tolerability and quality of life. The study is being conducted in several centers in Europe.
- **Phase 2 Study in Patients with Recurrent Glioblastoma (KING).** The primary goal of this Phase 2 study, known as the KING study, is to determine the anti-tumor activity of single agent Selinexor in up to 30 patients with relapsed glioblastoma (grade 4 glioma), as well as to document brain penetration of Selinexor and determine tolerability in this population. Eligible patients for this clinical trial have disease that has recurred after prior treatment with radiation therapy and temozolomide. Patients may also undergo surgery as required. The study is being conducted in Copenhagen, Boston and New York.

Planned Registration-Directed and Additional Study Initiations

Karyopharm has announced the planned initiation of five additional company-sponsored studies of Selinexor.

- **Acute Myeloid Leukemia (AML): Randomized, Registration-Directed Clinical Trial (KCP-330-008)** — Initiation expected during the second quarter of 2014.
- **Diffuse Large B-Cell Lymphoma (DLBCL): Randomized, Registration-Directed Clinical Trial (KCP-330-009)** — Initiation expected during the second half of 2014.
- **Richter’s Syndrome: Registration-Directed Clinical Trial (KCP-330-010)** — Initiation expected during the second quarter of 2014.
- **Squamous Cell Cancers of the Head and Neck, Lung or Esophagus (KCP-330-006)** — Initiation expected during the second quarter of 2014.
- **Metastatic Castration Resistant Prostate Cancer (KCP-330-007)** — Initiation expected during the second half of 2014.

Karyopharm also anticipates that approximately 20 investigator studies may begin in 2014, with Selinexor as a single agent therapy as well as in combination with other treatments.

About Selinexor

Selinexor (KPT-330) is a first-in-class, oral Selective Inhibitor of Nuclear Export (SINE) compound. Selinexor functions by binding with the nuclear export protein XPO1 (also called CRM1), leading to the accumulation of tumor suppressor proteins in the cell nucleus, which subsequently reinitiates and amplifies their tumor suppressor function. This is believed to lead to the selective induction of apoptosis in cancer cells, while largely sparing normal cells. Over 300 patients have been treated with Selinexor in Phase 1 and Phase 2 trials in advanced hematologic malignancies and solid tumors. Additional Phase 1 and Phase 2 studies are ongoing or currently planned and three registration-directed clinical trials in hematological indications are expected to begin enrollment during 2014. The latest clinical trial information for Selinexor is available at www.clinicaltrials.gov.

About Karyopharm Therapeutics

Karyopharm Therapeutics Inc. (Nasdaq:KPTI) is a clinical-stage pharmaceutical company focused on the discovery and development of novel first-in-class drugs directed against nuclear transport targets for the treatment of cancer and other major diseases. SINE compounds have shown biological activity in models of cancer, autoimmune disease, certain viruses, and wound-healing. Karyopharm was founded by Dr. Sharon Shacham and is located in Natick, Massachusetts.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Such forward-looking statements include those regarding the therapeutic potential of and potential clinical development plans for Karyopharm’s drug candidates, including the timing of initiation of certain trials and of the reporting of data from such trials. Such statements are subject to numerous important factors, risks and uncertainties that may cause actual events or results to differ materially from the company’s current expectations. For example, there can be no guarantee that any of Karyopharm’s SINE compounds, including Selinexor (KPT-330), or any other drug candidate that Karyopharm is developing will successfully complete necessary preclinical and clinical development

phases or that development of any of Karyopharm's drug candidates will continue. Further, there can be no guarantee that any positive developments in Karyopharm's drug candidate portfolio will result in stock price appreciation. Management's expectations and, therefore, any forward-looking statements in this press release could also be affected by risks and uncertainties relating to a number of other factors, including the following: Karyopharm's results of clinical trials and preclinical studies, including subsequent analysis of existing data and new data received from ongoing and future studies; the content and timing of decisions made by the U.S. Food and Drug Administration and other regulatory authorities, investigational review boards at clinical trial sites and publication review bodies; Karyopharm's ability to obtain and maintain requisite regulatory approvals and to enroll patients in its clinical trials; unplanned cash requirements and expenditures; development of drug candidates by Karyopharm's competitors for diseases in which Karyopharm is currently developing its drug candidates; and Karyopharm's ability to obtain, maintain and enforce patent and other intellectual property protection for any drug candidates it is developing. These and other risks are described under the caption "Risk Factors" in Karyopharm's Annual Report on Form 10-K for the year ended December 31, 2013, which is on file with the Securities and Exchange Commission (SEC), and in other filings that Karyopharm may make with the SEC in the future. Any forward-looking statements contained in this press release speak only as of the date hereof, and Karyopharm expressly disclaims any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise.

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917-392-3400

Karyopharm Therapeutics Inc.

(a development-stage company)

Unaudited Selected Consolidated Balance Sheet Information

(in thousands)

	<u>March 31,</u> <u>2014</u>	<u>December 31,</u> <u>2013</u>
Cash and cash equivalents	\$ 144,893	\$ 155,974
Prepaid expenses and other current assets	2,673	1,982
Property and equipment, net	322	240
Other assets	1,397	30
Total assets	\$ 149,285	\$ 158,226
Accounts payable and accrued expenses	\$ 4,823	\$ 2,908
Deferred revenue and other liabilities	362	384
Stockholders' equity	144,100	154,934
Total liabilities and stockholders' equity	\$ 149,285	\$ 158,226

Karyopharm Therapeutics Inc.

(a development-stage company)

Unaudited Condensed Consolidated Statement of Operation

(in thousands, except share and per share data)

	Three Months Ended March 31,	
	2014	2013
Revenue:		
Contract and grant revenue	\$ 171	\$ 233
Operating expenses:		
Research and development	10,979	4,965
General and administrative	2,904	879
Total operating expenses	<u>13,883</u>	<u>5,844</u>
Loss from operations	(13,712)	(5,611)
Interest income	18	—
Net loss	<u>\$ (13,694)</u>	<u>\$ (5,611)</u>
Net loss per share applicable to common stockholders—basic and diluted	<u>\$ (0.46)</u>	<u>\$ (2.52)</u>
Weighted-average number of common shares used in net loss per share applicable to common stockholders—basic and diluted	<u>29,606,683</u>	<u>2,225,596</u>