

Karyopharm Therapeutics Inc.  
Form 10-Q  
November 10, 2014  
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**UNITED STATES**  
**SECURITIES AND EXCHANGE COMMISSION**

Washington, D.C. 20549

**FORM 10-Q**

(Mark One)

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

For the quarterly period ended September 30, 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)

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

**85 Wells Avenue, 2nd Floor**

**Newton, MA**  
(Address of principal executive offices)

**02459**  
(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, 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

Accelerated filer

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

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 November 4, 2014 there were 32,700,563 shares of Common Stock, \$0.0001 par value per share, outstanding.



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Table of Contents**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)**

|  | September 30,<br>2014 | December 31,<br>2013 |
|--|-----------------------|----------------------|
| <b>Assets</b>  |                       |                      |
| Current assets:  |                       |                      |
| Cash and cash equivalents  | \$ 227,131            | \$ 155,974           |
| Prepaid expenses and other current assets  | 4,215                 | 1,982                |
| Total current assets   | 231,346               | 157,956              |
| Property and equipment, net  | 2,734                 | 240                  |
| Other assets   | 675                   | 30                   |
| Restricted cash  | 400                   |                      |
| Total assets   | \$ 235,155            | \$ 158,226           |
| <b>Liabilities and stockholders equity</b>   |                       |                      |
| Current liabilities:   |                       |                      |
| Accounts payable   | \$ 2,102              | \$ 1,740             |
| Accrued expenses   | 3,649                 | 1,168                |
| Deferred revenue   | 14                    | 79                   |
| Deferred rent  | 142                   |                      |
| Other current liabilities  | 327                   | 305                  |
| Total current liabilities  | 6,234                 | 3,292                |
| Deferred rent  | 1,117                 |                      |
| Total liabilities  | 7,351                 | 3,292                |
| 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; 32,614,387 and 29,587,258 shares issued and outstanding at September 30, 2014 and December 31, 2013, respectively | 3                     | 3                    |
| Additional paid-in capital   | 340,219               | 217,500              |

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|  |    |           |    |          |
|--|----|-----------|----|----------|
| Accumulated deficit                        |    | (112,418) |    | (62,569) |
| Total stockholders' equity                 |    | 227,804   |    | 154,934  |
| Total liabilities and stockholders' equity | \$ | 235,155   | \$ | 158,226  |

See accompanying notes to condensed consolidated financial statements.

Table of Contents**Karyopharm Therapeutics Inc.****CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS****(unaudited)****(in thousands, except share and per share amounts)**

|   | Three Months ended,<br>September 30, |            | Nine Months ended<br>September 30, |             |
|---|--------------------------------------|------------|------------------------------------|-------------|
|   | 2014                                 | 2013       | 2014                               | 2013        |
| Contract and grant revenue  | \$ 21                                | \$         | \$ 214                             | \$ 366      |
| Operating expenses:   |                                      |            |                                    |             |
| Research and development  | 15,951                               | 7,738      | 40,089                             | 18,763      |
| General and administrative  | 3,814                                | 1,583      | 10,028                             | 3,405       |
| Total operating expenses  | 19,765                               | 9,321      | 50,117                             | 22,168      |
| Loss from operations  | (19,744)                             | (9,321)    | (49,903)                           | (21,802)    |
| Interest income   | 20                                   |            | 54                                 | 1           |
| Net loss  | \$ (19,724)                          | \$ (9,321) | \$ (49,849)                        | \$ (21,801) |
| Net loss per share applicable to common stockholders basic and diluted  | \$ (0.61)                            | \$ (3.66)  | \$ (1.63)                          | \$ (9.11)   |
| Weighted-average number of common shares outstanding used in net loss per share applicable to common stockholders basic and diluted | 32,558,646                           | 2,544,587  | 30,619,074                         | 2,392,589   |
| Comprehensive loss  | \$ (19,724)                          | \$ (9,321) | \$ (49,849)                        | \$ (21,801) |

See accompanying notes to condensed consolidated financial statements.

Table of Contents**Karyopharm Therapeutics Inc.****CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS****(unaudited)****(in thousands)**

|   | <b>Nine Months ended September 30,</b> |             |
|---|--|-------------|
|   | <b>2014</b>                            | <b>2013</b> |
| <b>Operating activities</b>   |  |             |
| Net loss  | \$ (49,849)                            | \$ (21,801) |
| Adjustments to reconcile net loss to net cash used in operating activities: |  |             |
| Depreciation and amortization   | 191                                    | 106         |
| Noncash consulting expense  |  | 88          |
| Loss on disposal of fixed assets  | 50                                     |             |
| Stock-based compensation expense  | 9,649                                  | 1,769       |
| Changes in operating assets and liabilities:                                |  |             |
| Prepaid expenses and other current assets                                   | (2,233)                                | (95)        |
| Other non-current assets  | (645)                                  | (2,042)     |
| Accounts payable  | 362                                    | 673         |
| Accrued expenses and other liabilities                                      | 2,601                                  | 1,156       |
| Deferred revenue  | (65)                                   | (66)        |
| Deferred rent   | 1,259                                  |             |
| Net cash used in operating activities                                       | (38,680)                               | (20,212)    |
| <b>Investing activities</b>   |  |             |
| Purchases of property and equipment   | (2,735)                                | (25)        |
| Increase in restricted cash   | (400)                                  |             |
| Net cash used in investing activities                                       | (3,135)                                | (25)        |
| <b>Financing activities</b>   |  |             |
| Proceeds from the issuance of common stock, net of issuance costs           | 112,837                                | 340         |
| Proceeds from the exercise of stock options                                 | 135                                    |             |
| Proceeds from sale of convertible preferred stock, net of issuance costs    |  | 72,434      |
| Net cash provided by financing activities                                   | 112,972                                | 72,774      |
| Net increase in cash and cash equivalents                                   | 71,157                                 | 52,537      |
| Cash and cash equivalents at beginning of period                            | 155,974                                | 391         |
| Cash and cash equivalents at end of period                                  | \$ 227,131                             | \$ 52,928   |
| <b>Supplemental disclosure of non-cash financing activity</b>               |  |             |
| Issuance of preferred stock in satisfaction of preferred stock subscription | \$                                     | \$ 13,980   |

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. (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 and nine months ended September 30, 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 September 30, 2014 include the accounts of Karyopharm Therapeutics Inc. (a Delaware corporation), the accounts of Karyopharm Securities Corp. (KPSC, a wholly-owned Massachusetts corporation of the Company incorporated in December 2013), and the accounts of Karyopharm Europe GmbH (a wholly-owned German Limited Liability Company, incorporated in September 2014). At December 31, 2013, the consolidated financial statements also included the accounts of NPM Pharma Inc. (NPM, a wholly-owned Canadian corporation of the Company). As of March 31, 2014, NPM transferred its remaining assets and liabilities to Karyopharm Therapeutics Inc. Following the transfer, NPM was dissolved. The dissolution of NPM had no effect on the consolidated financial statements.

*Subsequent Events*

The Company considers events or transactions that occur after the balance sheet date but prior to the issuance of the financial statements to provide additional evidence relative to certain estimates or to identify matters that require additional disclosure. The Company has completed an evaluation of all subsequent events through the date of the filing of this Quarterly Report on Form 10-Q, and has determined that no events or transactions require recognition or disclosure.

## 2. Recently Issued Accounting Pronouncements

In May 2014, the Financial Accounting Standards Board ( FASB ) issued Accounting Standards Update ( ASU ) No. 2014-09, Revenue from Contracts with Customers ( ASU 2014-09 ), which supersedes the revenue recognition requirements in Accounting Standards Codification Topic 605, Revenue Recognition and most industry-specific guidance. The new standard requires that an entity recognize revenue to depict the transfer of promised goods or services to customers in an amount that reflects the consideration to which the company expects to be entitled in exchange for those goods or services. The update also requires additional disclosure about the nature, amount, timing and uncertainty of revenue and cash flows arising from customer contracts, including significant judgments and changes in judgments and assets recognized from costs incurred to obtain or fulfill a contract. ASU 2014-09 is effective for fiscal years, and interim periods within those years, beginning after December 15, 2016 and should be applied retrospectively to each prior reporting period presented or retrospectively with the cumulative effect of initially applying this update recognized at the date of initial application. The Company has not determined yet the potential effects of the adoption of this standard on its consolidated financial position, results of operations or cash flows.

On June 10, 2014, the FASB issued ASU 2014-10, which simplifies financial reporting for development stage entities by eliminating requirements specific to development stage entities. As a result, entities in a development stage will no longer need to present inception-to-date information about income statement line items, cash flows, and equity transactions. Instead, the new guidance clarifies how these entities should tailor existing disclosures to explain the risks and uncertainties related to their activities. This update is effective for annual periods beginning after December 15, 2014, and early application is permitted for any annual or interim period for which the entity's financial statements have not yet been issued. The Company adopted this guidance prior to issuing the interim financial statements for the three and six months ended June 30, 2014. The adoption of ASU 2014-10 impacted disclosure only and did not have any impact on the Company's financial position or results of operations.

The Company did not adopt any new accounting pronouncements during the nine months ended September 30, 2014 that had an effect on the Company's condensed consolidated financial statements, except for the adoption of ASU2014-10 discussed above.

## 3. 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 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 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. The following table presents information about the Company's financial assets that have been measured at fair value at September 30, 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<br>in active<br>markets<br>(Level 1) | Significant<br>other<br>observable<br>inputs<br>(Level 2) | Significant<br>unobservable<br>inputs<br>(Level 3) |
|--|------------|--|---|--|
| <b>Financial assets</b>                          |            |  |   |  |
| Money Market Funds, included in cash equivalents | \$ 219,067 | \$ 219,067   | \$  | \$   |

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<br>in active<br>markets<br>(Level 1) | Significant<br>other<br>observable<br>inputs<br>(Level 2) | Significant<br>unobservable<br>inputs<br>(Level 3) |
|--|------------|--|---|--|
| <b>Financial assets</b>                          |            |  |   |  |
| Money Market Funds, included in cash equivalents | \$ 155,765 | \$ 155,765   | \$  | \$   |

**4. Property and Equipment, net**

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

|  | Estimated Useful<br>Life Years         | September 30,<br>2014 | December 31,<br>2013 |
|--|--|-----------------------|----------------------|
| Laboratory equipment                           | 4                                      | \$ 434                | \$ 328               |
| Furniture and fixtures                         | 5                                      | 60                    | 98                   |
| Office and computer equipment                  | 3                                      | 173                   | 85                   |
| Leasehold improvements                         | Lesser of useful life<br>or lease term | 2,430                 | 79                   |
|  |  | 3,097                 | 590                  |
| Less accumulated depreciation and amortization |  | (363)                 | (350)                |
|  |  | \$ 2,734              | \$ 240               |

**5. Accrued Expenses**

Accrued expenses consist of the following (in thousands):

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|                                    | September 30,<br>2014 |       | December 31,<br>2013 |       |
|------------------------------------|-----------------------|-------|----------------------|-------|
| Research and development costs     | \$                    | 1,660 | \$                   | 698   |
| Payroll and employee-related costs |                       | 1,228 |                      | 100   |
| Professional fees                  |                       | 167   |                      | 215   |
| Other                              |                       | 594   |                      | 155   |
|                                    | \$                    | 3,649 | \$                   | 1,168 |

**6. 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, 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.

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The following potentially dilutive securities were excluded from the calculation of diluted net loss per share due to their anti-dilutive effect at September 30, 2014 and 2013:

|                             | September 30, |            |
|-----------------------------|---------------|------------|
|                             | 2014          | 2013       |
| Convertible preferred stock |               | 19,114,241 |
| Outstanding stock options   | 2,942,927     | 1,775,593  |
| Unvested restricted stock   | 78,015        | 224,019    |

**7. 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 also 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, and is 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 September 30, 2014 and changes during the nine months ended September 30, 2014 is as follows:

|                                   | Shares  | Weighted-<br>average<br>purchase price<br>per share |      |
|-----------------------------------|---------|---|------|
| Unvested at December 31, 2013     | 9,943   | \$  | 0.26 |
| Vested                            | (4,261) |   | 0.26 |
| Unvested at September 30, 2014(1) | 5,682   | \$  | 0.26 |

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(1) Excludes 72,333 shares of unvested restricted stock remaining from the early exercise of stock options.

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As of September 30, 2014, there was \$199 of total unrecognized stock-based compensation expense related to unvested restricted stock. The expense is expected to be recognized over a weighted average period of 0.9 years.

### Stock options

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

|   | Shares    | Weighted-<br>average<br>price<br>per share | Weighted-<br>average<br>remaining<br>contractual<br>term (years) | Aggregate<br>intrinsic<br>value<br>(in thousands) |
|---|-----------|--|--|---|
| Outstanding at December 31, 2013                  | 2,410,522 | \$ 7.85                                    | 9.3  | \$ 36,717   |
| Granted   | 627,600   | 38.73                                      |  |   |
| Exercised   | (94,342)  | 1.43                                       |  |   |
| Canceled  | (853)     | 1.49                                       |  |   |
| Outstanding at September 30, 2014                 | 2,942,927 | \$ 14.64                                   | 8.9  | \$ 62,329   |
| Exercisable at September 30, 2014                 | 668,114   | \$ 2.35                                    | 7.7  | \$ 21,776   |
| Vested and expected to vest at September 30, 2014 | 2,749,715 | \$ 14.53                                   | 8.8  | \$ 58,507   |



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As of September 30, 2014, there was \$30,482 of total unrecognized stock-based compensation expense related to stock options. The expense is expected to be recognized over a weighted average period of 3.2 years.

In 2014 certain individuals changed status from employees to non-employees. The outstanding stock option awards for these individuals continued to vest under the original vesting terms of the awards as the individuals continued to provide service to the Company as consultants. In addition, in August 2014, the Company modified a stock option grant for one of these individuals to accelerate vesting of one of his stock option awards in connection with his separation agreement. The Company recorded expense of \$1,100 in the third quarter of 2014 for the grants outstanding to these individuals. As of September 30, 2014, there was \$466 of unrecognized compensation expense related to these awards that is expected to be recognized in the fourth quarter of 2014.

## **Employee Stock Purchase Plan**

The Company has an Employee Stock Purchase Plan ( ESPP ) that permits eligible employees to enroll in a twelve-month offering period comprising two six-month purchase 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 day of the applicable six-month offering period, or the last day of the applicable six-month purchase 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 shareholders approved an increase in the number of shares of common stock authorized for issuance pursuant to the ESPP. There are 242,424 shares of common stock authorized for issuance pursuant to the ESPP, and no shares have been issued to employees under the ESPP to date. As of September 30, 2014, there was \$10 of total unrecognized stock-based compensation expense related to the ESPP. The expense is expected to be recognized over a period of one month.

## **8. 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 commenced in May 2014 and expires in October 2021. The Company may extend the lease term for one additional five year period. Pursuant to the lease agreement, the Company is obligated to make aggregate rent payments of \$5,600 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, which base rent rises periodically until it reaches approximately \$898. 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 payment. The Company has recorded deferred rent on the condensed consolidated balance sheet at September 30, 2014, accordingly. 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 \$1,000 which was incurred through September 30, 2014, was recorded as a leasehold improvement and deferred rent, and will be recorded as a reduction to rent expense ratably over the lease term. The balance from the leasehold improvement incentives is included in deferred rent on the balance sheets. The Company has provided a security deposit in the form of a cash-collateralized letter of credit in the amount of \$400, which amount may be reduced to \$200 in January 2018. The amount is classified as restricted cash on the condensed consolidated balance sheet.

## **9. Equity**

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In July 2014, the Company completed a public offering of its common stock, which resulted in the sale of 2,447,247 shares of its common stock at a public offering price of \$42.50 per share. Also, in July 2014, the Company issued 397,087 shares of its common stock upon exercise by the underwriters of their option to purchase additional shares. The Company received net proceeds of approximately \$112,800, after deducting underwriting discounts, commissions and expenses payable by the Company of \$794.

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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 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, 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, 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.

**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 are developing wholly-owned novel, small molecule, **S**elective **I**nhibitor of **N**uclear **E**xport, 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, we have administered Selinexor to over 450 patients across Phase 1 and Phase 2 clinical trials in hematologic and solid tumor indications. Evidence of anti-cancer activity has been observed in many patients and Selinexor has been

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sufficiently well-tolerated to allow many of these patients to remain on therapy for prolonged periods, including several who have remained on study for over 12 months. In June 2014, we initiated a registration-directed clinical trial in older patients with acute myeloid leukemia, or AML, and in November 2014, we initiated a registration-directed clinical trial in patients with Richter's Transformation. We plan to initiate a registration-directed clinical trial in patients with diffuse large B-cell lymphoma, or DLBCL, in 2014. We also plan to initiate up to two additional registration-directed clinical trials in hematological or solid tumor indications during the first half of 2015, including a potential registration-directed clinical trial in patients with multiple myeloma. To our knowledge, no other XPO1 inhibitors are in clinical development at the present time.

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 public offerings of our common stock.

As of September 30, 2014, we had an accumulated deficit of \$112.4 million. We had net losses of \$49.9 million and \$21.8 million for the nine months ended September 30, 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;
- 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;

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- 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.

There were no changes to 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 in the three and nine months ended September 30, 2014. It is important that the discussion of our 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 September 30, 2014 and September 30, 2013**

|                            | Three Months Ended September 30, |            |
|----------------------------|----------------------------------|------------|
|                            | 2014                             | 2013       |
|                            | (in thousands)                   |            |
| Contract and grant revenue | \$ 21                            | \$         |
| Operating expenses:        |                                  |            |
| Research and development   | 15,951                           | 7,738      |
| General and administrative | 3,814                            | 1,583      |
| Loss from operations       | (19,744)                         | (9,321)    |
| Interest income            | 20                               |            |
| Net loss                   | \$ (19,724)                      | \$ (9,321) |

*Contract and Grant Revenue.* We recognize revenue pursuant to a sponsored research agreement. Contract and grant revenue for the three months ended September 30, 2014 ( the 2014 Quarter ) was less than \$0.1 million compared to none for the three months ended September 30, 2013 ( the 2013 Quarter ). The increase in revenue was the result of recognizing revenue pursuant to grant funding during the 2014 Quarter.

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*Research and Development Expense.* Research and development expense increased by approximately \$8.3 million to approximately \$16.0 million in the 2014 Quarter from approximately \$7.7 million in the 2013 Quarter. The increase is primarily related to:

- an increase of approximately \$4.0 million in clinical trial costs, primarily related to an increase of approximately \$2.2 million in Contract Research Organization ( CRO ) costs and an increase of \$2.1 million in costs related to the manufacture of Selinexor, partially offset by a decrease of approximately \$0.3 million in costs related to the manufacture of Verdinexor,
- an increase of approximately \$1.8 million in personnel costs, primarily due to increased headcount and an increase of \$0.9 million 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 approximately \$1.6 million in preclinical efficacy and toxicology study costs, and
- an increase of approximately \$0.4 million in occupancy costs.

*General and Administrative Expense.* General and administrative expense increased by approximately \$2.3 million to approximately \$3.8 million for the 2014 Quarter from approximately \$1.6 million for the 2013 Quarter. The increase is primarily related to:

- an increase of approximately \$1.2 million in personnel costs, primarily due to increased headcount and an increase of approximately \$1.0 million in stock-based compensation expense related to equity awards granted to personnel, primarily related to the higher fair value of our common stock,
- a net increase of approximately \$0.8 million in consulting fees, primarily related to an increase in stock-based compensation expense related to equity awards granted to consultants, primarily due to the higher fair value of our common stock, which is partially offset by decreases in consulting fees related to finance and business development,
- an increase of approximately \$0.2 million in professional fees, primarily related to higher costs related to being a public company, including higher public and investor relations costs, higher legal fees for protecting our intellectual property, and higher corporate legal fees and audit fees, and
- an increase of approximately \$0.2 million in insurance expense, primarily due to our becoming a publicly traded company.

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*Interest income.* Interest income was less than \$0.1 million for all periods presented.

**Comparison of the Nine Months Ended September 30, 2014 and September 30, 2013**

|                            | <b>Nine Months Ended September 30,</b> |             |
|----------------------------|--|-------------|
|                            | <b>2014</b>                            | <b>2013</b> |
|                            | <b>(in thousands)</b>                  |             |
| Contract and grant revenue | \$ 214                                 | \$ 366      |
| Operating expenses:        |  |             |
| Research and development   | 40,089                                 | 18,763      |
| General and administrative | 10,028                                 | 3,405       |
| Loss from operations       | (49,903)                               | (21,802)    |
| Interest income            | 54                                     | 1           |
| Net loss                   | \$ (49,849)                            | \$ (21,801) |

*Contract and Grant Revenue.* We recognize revenue pursuant to a sponsored research agreement. Contract and grant revenue for the nine months ended September 30, 2014 ( the 2014 Period ) was \$0.2 million compared to \$0.3 million for the nine months ended September 30, 2013 ( the 2013 Period ). The decrease in revenue was the result of recognizing less revenue pursuant to grant funding during the 2014 Period.

*Research and Development Expense.* Research and development expense increased by approximately \$21.3 million to \$40.1 million in the 2014 Period from \$18.8 million in the 2013 Period. The increase is primarily related to:

- an increase of approximately \$6.8 million in clinical trial costs, primarily related to an increase of approximately \$4.5 million in costs to CROs, and an increase of approximately \$4.1 million in costs related to the manufacture of Selinexor, partially offset by a decrease of \$1.8 million related to the manufacture of Verdinexor,
- an increase of approximately \$4.7 million in personnel costs, primarily due to increased headcount and an increase of approximately \$2.3 million 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 approximately \$3.8 million in consulting fees, primarily due to an increase of approximately \$2.2 million paid to consultants and an increase of approximately \$1.6 million 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 approximately \$3.4 million in preclinical efficacy and toxicology study costs,
- an increase of approximately \$2.0 million in discovery work, including preclinical studies and screening, and offset by
- a decrease of approximately \$0.9 million in costs related to our clinical trials of Verdinexor in pet dogs.

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*General and Administrative Expense.* General and administrative expense increased by approximately \$6.6 million to approximately \$10.0 million for the 2014 Period from approximately \$3.4 million for the 2013 Period. The increase is primarily related to:

- an increase of approximately \$3.1 million in personnel costs, primarily due to increased headcount and an increase of approximately \$2.2 million in stock-based compensation expense related to equity awards granted to personnel, primarily related to the higher fair value of our common stock.
- a net increase of approximately \$1.9 million in consulting fees, primarily related to an increase in stock-based compensation expense related to equity awards granted to consultants, primarily due to the higher fair value of our common stock, partially offset by decreases in costs for consultants related to finance and business development
- an increase of approximately \$1.1 million in professional fees, primarily related to higher legal fees for protecting our intellectual property, higher corporate legal fees, and higher public and investor relations fees and other fees to operate as a public company,
- an increase of approximately \$0.5 million in insurance expense, primarily due to our becoming a publicly traded company, and
- an increase of approximately \$0.2 million in state tax expenses.

*Interest income.* Interest income was less than \$0.1 million for all periods presented.

### **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 and follow-on offering.

As of September 30, 2014, we had \$227.1 million in cash and cash equivalents. We primarily invest our cash and cash equivalents in money market funds. In July 2014, we completed a public offering of 2,844,334 shares of our common stock at a public offering price of \$42.50 per



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share. We received net proceeds of approximately \$112.8 million, after deducting underwriting discounts, commissions and expenses payable by us.

**Cash flows**

The following table provides information regarding our cash flows:

|   | Nine Months Ended September 30, |             |
|---|---------------------------------|-------------|
|   | 2014                            | 2013        |
|   | (in thousands)                  |             |
| Net cash used in operating activities     | \$ (38,680)                     | \$ (20,212) |
| Net cash used in investing activities     | (3,135)                         | (25)        |
| Net cash provided by financing activities | 112,972                         | 72,774      |
| Net increase in cash and cash equivalents | \$ 71,157                       | \$ 52,537   |

*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 Period compared to the 2013 Period 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 related to Selinexor.

*Investing activities.* The cash used in investing activities for the 2014 Period reflects an increase of \$0.4 million in restricted cash related to a facility lease and the purchase of \$2.7 million of property and equipment related to the renovation of and relocation to new office and laboratory space. There was less than \$0.1 million in investing activities in the 2013 period.

*Financing activities.* The cash provided by financing activities for the 2014 Period reflects net proceeds from the sale of common stock as part of a public offering of our common stock in July 2014, and the proceeds from the exercise of stock options. The cash provided by financing activities in the 2013 Period was primarily from the sale of preferred stock.

**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

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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 the second half of 2017. We expect to finish 2014 with greater than \$200 million in cash and cash equivalents. 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;
- 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

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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***

Our contractual obligations were updated in the March 31, 2014 Form 10-Q for the execution of the lease of our corporate headquarters.

There have been no material changes to our contractual obligations during the three months ended September 30, 2014.

**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 \$227.1 as of September 30, 2014, consisting of cash and 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 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 are also exposed to market risk related to change in foreign currency exchange rates. We contract with CROs and Contract Manufacturing Organizations ( 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. At this time, an immediate 10% change in currency exchange rates would not have a material effect on our financial position, results of operations or cash flows.

**Item 4. Controls and Procedures.**

**Evaluation of Disclosure Controls and Procedures**

Our management, with the participation of our Chief Executive Officer and Executive Vice President and Chief Financial Officer, evaluated the effectiveness of our disclosure controls and procedures as of September 30, 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 September 30, 2014, our Chief Executive Officer and Executive Vice President and Chief Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

#### **Changes in Internal Control Over Financial Reporting**

No change in our internal control over financial reporting occurred during the fiscal quarter ended September 30, 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

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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 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 or net offering proceeds 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 September 30, 2014, we have used approximately \$47.4 million of such net offering proceeds to fund the continued clinical development of our lead drug candidate, Selinexor, the preclinical development of our drug candidates for anti-inflammatory, viral and wound-healing indications, the discovery, research and preclinical development 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

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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.**

None.

**Item 6. Exhibits.**

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

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**SIGNATURES**

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

**KARYOPHARM THERAPEUTICS INC.**

Date: November 10, 2014

By:

/s/ MICHAEL KAUFFMAN  
Michael Kauffman, M.D., Ph.D.  
*Chief Executive Officer*  
*(Principal executive officer)*

Date: November 10, 2014

By:

/s/ JUSTIN A. RENZ  
Justin A. Renz  
*Executive Vice President and Chief Financial Officer*  
*(Principal financial and accounting officer)*

Table of Contents**EXHIBIT INDEX**

| <b>Exhibit Number</b> | <b>Description of Exhibit</b>   | <b>Form</b> | <b>File Number</b> | <b>Incorporated by Reference<br/>Date of Filing</b> | <b>Exhibit Number</b> | <b>Filed<br/>Herewith</b> |
|-----------------------|---|-------------|--------------------|---|-----------------------|---------------------------|
| 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                         |
| 101.INS               | Instance Document   |             |                    |   |                       | X                         |
| 101.SCH               | Scheme Document   |             |                    |   |                       | X                         |
| 101.CAL               | Calculation Linkbase Document   |             |                    |   |                       | X                         |
| 101.DEF               | Definition Linkbase Document  |             |                    |   |                       | X                         |
| 101.LAB               | Labels Linkbase Document  |             |                    |   |                       | X                         |
| 101.PRE               | Presentation Linkbase Document  |             |                    |   |                       | X                         |