



May 7, 2014

## **Karyopharm Therapeutics Reports First Quarter Financial Results for 2014**

NATICK, Mass., May 7, 2014 (GLOBE NEWSWIRE) -- 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/m<sup>2</sup> twice weekly. This corresponds to 80-140 mg per dose. Once weekly dosing with Selinexor is currently being evaluated at doses of 80 mg/m<sup>2</sup> 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  $\geq$  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](http://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.

**Karyopharm Therapeutics Inc.**  
**(a development-stage company)**

**Unaudited Selected Consolidated Balance Sheet Information**

(in thousands)

	<b>March 31, 2014</b>	<b>December 31, 2013</b>
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	<u>1,397</u>	<u>30</u>
<b>Total assets</b>	<b>\$149,285</b>	<b>\$158,226</b>
Accounts payable and accrued expenses	\$4,823	\$2,908
Deferred revenue and other liabilities	362	384
Stockholders' equity	<u>144,100</u>	<u>154,934</u>
<b>Total liabilities and stockholders' equity</b>	<b>\$149,285</b>	<b>\$158,226</b>

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**Unaudited Condensed Consolidated Statement of Operation**

(in thousands, except share and per share data)

	<b>Three Months Ended March 31,</b>	
	<b>2014</b>	<b>2013</b>
Revenue:		
Contract and grant revenue	\$171	\$233

Operating expenses:		
Research and development	10,979	4,965
General and administrative	<u>2,904</u>	<u>879</u>
Total operating expenses	<u>13,883</u>	<u>5,844</u>
Loss from operations	(13,712)	(5,611)
Interest income	<u>18</u>	<u>—</u>
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>

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Source: Karyopharm Therapeutics

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