

# Karyopharm Therapeutics Reports 2013 Year-End Financial Results and Provides Update on Clinical Development Plans

NATICK, Mass., March 5, 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, reported financial results for the year ended December 31, 2013, and also provided an update on its clinical development plans.

"2013 has been a very important year for Karyopharm, highlighted by robust clinical development activity focused on our novel, first in class, orally active selective inhibitors of nuclear export compounds, also called SINEs," said Michael Kauffman, CEO of Karyopharm Therapeutics. "We have moved rapidly from discovery to the clinic. In 2014, we plan to initiate three registration-directed trials in hematological indications and to continue to broaden our solid tumor development activity. This foundation positions us to continue our broad-based development plan to assess the breadth of this novel anti-cancer mechanism."

## Full Year 2013 Financial Results

Cash and cash equivalents as of December 31, 2013, totaled \$156.0 million compared with \$391,000 as of December 31, 2012. The increase reflects gross proceeds of \$67.2 million from private placements prior to Karyopharm's initial public offering and gross proceeds of \$125.1 million from the initial public offering.

For the year ended December 31, 2013, research and development expense was \$28.5 million compared to \$14.1 million for the year ended December 31, 2012. For the year ended December 31, 2013, general and administrative expense was \$5.9 million compared to \$2.4 million for the year ended December 31, 2012. 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 \$33.9 million, or \$5.59 per share, for the year ended December 31, 2013, compared to a net loss of \$15.9 million, or \$8.95 per share, for the year ended December 31, 2012. The weighted-average shares outstanding used to compute earnings per share was 6,067,679 for the year ended December 31, 2013, compared to 1,775,323 for the year ended December 31, 2012. Net loss includes stock-based compensation expense of \$3.8 million and \$653,000 for the years ended December 31, 2013 and 2012, 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 Update

The company discussed the status of three Phase 1 clinical trials with its lead drug candidate, Selinexor (KPT-330), a first in class, oral Selective Inhibitor of Nuclear Export (SINE) compound, as well as near-term study initiations directed at potential registration.

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. To date, over 240 patients have been treated with Selinexor, all have whom entered the trials with progressive disease relapsed or refractory to essentially all available classes of agents. Dose escalation began at 3mg/m<sup>2</sup> and has now cleared 60 mg/m<sup>2</sup> in hematological indications and 65 mg/m<sup>2</sup> in solid tumors. A growing number of patients have been treated for over a year with no clinically significant cumulative toxicities, and preliminary evidence of broad anti-cancer activity has been observed. The use of appetite stimulants in all patients who begin therapy with Selinexor has been observed to improve tolerability.

Karyopharm plans to initiate two randomized, registration-directed trials in relapsed hematologic malignancies this year, one in Acute Myelogenous Leukemia (AML) and the other in diffuse large B-cell lymphoma (DLBCL).

The first of the two planned randomized trials will be in patients over 60 years of age with AML in first relapse, who are not candidates for intensive chemotherapy or transplantation. The primary endpoint is overall survival. The Phase 2 trial is expected to enroll about 150 patients who will be randomized 2:1 with Selinexor versus the treating physicians' choice. The physician's choice includes best supportive care (BSC includes transfusions, antibiotics and growth factors as appropriate), or BSC + low dose Ara-C, or BSC+ a hypomethylating agent (azacitidine or decitabine). Selinexor will be given at a dose of 55 mg/m<sup>2</sup>, administered orally two times per week. The trial is expected to begin in the first half of this year and is expected to take two years to complete.

The second of the two planned randomized trials will be in patients with DLBCL who have progressed after at least two lines of chemotherapy and anti-CD20 monoclonal antibodies. The primary endpoint is progression free survival (or PFS). The trial is expected to enroll approximately 300 DLBCL patients who will be randomized 2:1 with Selinexor versus single agent, physician's choice chemotherapy. Selinexor will be given at a dose of 60 mg/m<sup>2</sup>, administered orally two times per week. The trial is expected to begin in late summer 2014.

Based on preliminary data and clinical observations, Karyopharm said it expects to initiate additional clinical trials in one or more indications, including:

- Richter's syndrome, in patients whose disease has relapsed after initial treatment, typically multi-agent chemoimmunotherapy
- Gynecological malignancies in patients with ovarian, cervical and uterine carcinomas
- Advanced head and neck and lung cancer
- Hormone and/or chemotherapy refractory metastatic prostate cancer
- Relapsed glioblastoma multiforme

Karyopharm also commented that it anticipates that approximately 20 investigator studies may begin in 2014, some with Selinexor as the single agent therapy and others in combination with other treatments.

## Conference Call Details

Karyopharm will host a conference call on Wednesday, March 5, 2014, at 8:00 a.m. Eastern Time to review Karyopharm's 2013 financial results and provide an update on its development programs.

To access the live conference call via phone, please dial (855) 437-4406 from the United States and Canada or (484) 756-4292 internationally. The conference ID is 6470393.

To access the live and subsequently archived audio webcast, visit the Investors section of the Karyopharm website at [www.karyopharm.com](http://www.karyopharm.com). A replay of the webcast will be archived on the Company's website for 30 days following the call.

#### About Selinexor

Selinexor (KPT-330) is a first-in-class, oral Selective Inhibitor of Nuclear Export (SINE) compound that is undergoing Phase 1 clinical trials in patients with advanced hematologic malignancies (NCT01607892), solid tumors (NCT01607905), and sarcomas (NCT01896505). 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.

#### About Karyopharm

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. Karyopharm's SINE compounds function by binding with the XPO1, which prevents the export of various proteins out of the nucleus. 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 SINE compounds and PAK4 inhibitors, including the timing of initiation of certain trials and of the reporting of data from such trials and from ongoing 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, including PAK4 inhibitors, 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 Quarterly Report on Form 10-Q for the quarter ended September 30, 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 Condensed Consolidated Balance Sheets

(in thousands)

|   | As of December 31, |          |
|---|--------------------|----------|
|   | 2013               | 2012     |
| Cash and cash equivalents   | \$155,974          | \$391    |
| Prepaid expenses and other current assets   | 1,982              | 563      |
| Property and equipment, net   | 240                | 327      |
| Other assets  | 30                 | 30       |
| Total assets  | \$158,226          | \$1,311  |
| Accounts payable and accrued expenses   | \$2,908            | \$1,840  |
| Deferred revenue and other liabilities  | 384                | 90       |
| Preferred stock subscription  | --                 | 8,980    |
| Convertible preferred stock   | --                 | 18,278   |
| Stockholders' equity (deficit)  | 154,934            | (27,877) |
| Total liabilities, convertible preferred stock and stockholders' equity (deficit) | \$158,226          | \$1,311  |

Karyopharm Therapeutics Inc.

(A Development-Stage Company)

Unaudited Condensed Consolidated Statement of Operation

(in thousands, except share and per share data)

|                            | Year ended December 31, |       |
|----------------------------|-------------------------|-------|
|                            | 2013                    | 2012  |
| Revenue:                   |                         |       |
| Contract and grant revenue | \$387                   | \$634 |

|   |            |            |
|---|------------|------------|
| Operating expenses:   |            |            |
| Research and development  | 28,452     | 14,095     |
| General and administrative  | 5,885      | 2,429      |
| Total operating expenses  | 34,337     | 16,524     |
| Loss from operations  | (33,950)   | (15,890)   |
| Interest income   | 3          | 2          |
| Net loss  | (\$33,947) | (\$15,888) |
| Net loss per share applicable to common stockholders-basic and diluted  | (\$5.59)   | (\$8.95)   |
| Weighted-average number of common shares used in net loss per share applicable to common stockholders-basic and diluted | 6,067,679  | 1,775,323  |

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<https://investors.karyopharm.com/2014-03-05-Karyopharm-Therapeutics-Reports-2013-Year-End-Financial-Results-and-Provides-Update-on-Clinical-Development-Plans>