May 11, 2015

Karyopharm Reports First Quarter 2015 Financial Results and Highlights Recent Progress

Positive Data in Difficult to Treat Cancers With Significant Unmet Need Further Supports Broad Clinical Development Campaign for Selinexor

Conference Call Scheduled for Today at 8:30 a.m. ET

NEWTON, Mass., May 11, 2015 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a clinical-stage pharmaceutical company, today reported financial results for the first quarter 2015 and commented on recent accomplishments and clinical development plans for selinexor, its lead product candidate.

"Karyopharm and its collaborators presented a comprehensive body of data across its oncology pipeline, including a total of twenty-one presentations at the 2015 Annual Meeting of the American Association for Cancer Research," said Michael G. Kauffman, MD, PhD, Chief Executive Officer of Karyopharm. "These data further demonstrated the role XPO1 inhibition in difficult to treat cancers with limited treatment options, including preclinical data describing the robust activity of selinexor alone and in combination with novel and standard of care therapies across a variety of hematologic and solid tumor cancers. Taken together, these data further support our ongoing broad clinical development plans for selinexor."

Conference Call Information:

To access the conference call, please dial (855) 437-4406 (US) or (484) 756-4292 (international) at least five minutes prior to the start time and refer to conference ID 41661952. A live audio webcast of the call will be available under "Events & Presentations" in the Investor section of Karyopharm's website, http://www.karyopharm.com/, approximately two hours after the event.

Recent Corporate Accomplishments:

- Appointed Mikael Dolsten, MD, PhD, President of Worldwide Research and Development of Pfizer Inc., to Karyopharm's board of directors.

Scientific Presentations and Publications:

- Presented positive data on Karyopharm's oncology pipeline at the 2015 Annual Meeting of the American Association for Cancer Research (AACR) including 21 presentations with preclinical data for selinexor (KPT-330) and the role of XPO1 in cancer and on Karyopharm's novel oral PAK4 allosteric modulators (PAMs).
  - Data presented for selinexor included mechanisms of drug action, specificity and molecular mechanisms leading to tumor radio-sensitization and to selective tumor and cancer stem cell killing. Data that expanded upon the understanding of selinexor's activity in hematologic malignances including non-Hodgkin's lymphoma, acute myeloid leukemia and multiple myeloma, and in solid tumors including sarcoma, mesothelioma, ovarian and non-small cell lung cancers, as well as in pediatric tumors including neuroblastoma, were also presented.
  - Two late-breaking abstracts representing company and investigator-sponsored preclinical studies were presented demonstrating the potential role of XPO1 inhibition in two difficult to treat tumor types for which current therapies provide minimal benefit. These data included potent anti-cancer effects of selinexor both alone and in combination with chemotherapy in double- and triple-hit diffuse large B-cell lymphoma, or DLBCL, patient cell lines consistent with selinexor's activity in Karyopharm's ongoing Phase 1 clinical trial in hematologic malignancies. In addition, the combination of selinexor and olaparib, an approved PARP inhibitor, showed synergistic activity in models of triple-negative breast cancer, supporting further investigation of selinexor in solid tumors.
  - Data related to the intercellular effects of PAMs on K-Ras and WNT/ß-catenin signaling pathways and the characterization of single-agent antitumor activity were also presented.
- Karyopharm plans to present solid tumor data on single agent selinexor at the upcoming 2015 American Society of Clinical Oncology (ASCO) annual meeting being held May 29 to June 2, 2015 in Chicago. Data to be presented include Phase 2 clinical updates in both gynecologic cancers and recurrent glioblastoma, as well as Phase 1b clinical data in advanced sarcomas and Phase 1 clinical data in Asian patients with advanced solid and hematological cancers.

Regulatory and Intellectual Property Updates:

- Granted U.S. patent for Karyopharm's lead product candidate, selinexor (KPT-330), a first-in-class, oral SINE™ compound. This patent, which will expire in 2032 absent any patent term extensions, covers the composition-of-matter of selinexor, as well as certain other compositions and related methods.
- Received U.S. patent allowance covering composition of matter for KPT-350, an oral SINE™ compound being develope for the treatment of inflammatory and autoimmune diseases. Once issued, this patent will provide patent protection for KPT-350 and pharmaceutical compositions comprising KPT-350 through 2033.

Clinical Development Plans:

- Karyopharm is actively enrolling patients in three registration-directed clinical studies evaluating selinexor: one in older patients with relapsed/refractory AML (SOPRA study), the second in patients with relapsed/refractory DLBCL (SADAL study) and the third in patients with relapsed/refractory Richter's transformation (SIRRT study). Preliminary top-line data from all three studies are anticipated in the second half of 2016.
- Karyopharm plans to initiate a single-arm trial in multiple myeloma called STORM, for **S**elinexor **T**reatment **o**f **R**efractory **M**yeloma, in the second quarter of 2015, which will initially include 80 patients. If the data from the initial 80 patients is promising, the study may be expanded to potentially support accelerated approval.
- Karyopharm is also actively enrolling patients in four Phase 2 solid tumor studies evaluating selinexor in gynecologic malignancies (SIGN Study), glioblastoma multiforme (KING Study), metastatic prostate cancer (SHIP Study) and squamous head and neck, lung and esophageal cancers (STARRS Study). Karyopharm will present interim data from SIGN and KING at ASCO.
- Karyopharm plans to initiate a registration-directed clinical trial of selinexor to treat liposarcoma in the second half of 2015. Accrual to Karyopharm's Phase 1b clinical trial in sarcomas, including liposarcoma, is nearly complete.
- In addition, a number of investigator-sponsored (ISTs) or company-sponsored clinical studies evaluating the potential of selinexor in combination with either chemotherapy or targeted agents are currently ongoing or planned.
- Karyopharm also plans to initiate a Phase 1 clinical study of verdinexor (KPT-335), an oral SINE™ compound closely related to selinexor that is being studied as a potential therapy for viral indications. This randomized, double-blind, placebo-controlled, dose-escalating trial, which has been designed to evaluate the safety and tolerability of verdinexor in healthy adult subjects, is expected to initiate in the second quarter of 2015 and will be conducted in Australia.

First Quarter March 31, 2015 Financial Results

Cash, cash equivalents and investments as of March 31, 2015, including restricted cash, totaled $285.3 million, compared to $214.8 million as of December 31, 2014. Karyopharm raised $90.8 million in a common stock follow-on offering which closed in January 2015.

For the quarter ended March 31, 2015, research and development expense was $20.8 million compared to $11.0 million for the quarter ended March 31, 2014. For the quarter ended March 31, 2015, general and administrative expense was $5.4 million compared to $2.9 million for the quarter ended March 31, 2014. The increase in research and development expenses resulted primarily from the increase in expenses related to the continued clinical development of selinexor. The increase in general and administrative expense resulted primarily from the costs of being a public company and an increase in stock-based compensation.

Karyopharm reported a net loss of $26.1 million, or $0.74 per share, for the quarter ended March 31, 2015, compared to a net loss of $13.7 million, or $0.46 per share, for the quarter ended March 31, 2014. Net loss includes stock-based compensation expense of $3.7 million and $2.8 million for the quarters ended March 31, 2015 and March 31, 2014, respectively.

Financial Outlook

Based on current operating plans, Karyopharm expects that its existing cash and cash equivalents will fund its research and development programs and operations into 2018, including moving registration-directed clinical studies to their next data inflection points. Karyopharm expects to end 2015 with greater than $200 million in cash, cash equivalents and investments.

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. Karyopharm's SINE™ compounds function by binding to and inhibiting the nuclear export protein XPO1 (or CRM1). addition to single-agent activity against a variety of different human cancers, SINE™ compounds have also shown biological activity in models of cancer, inflammation, autoimmune disease, certain viruses, and wound-healing. Karyopharm was founded by Dr. Sharon Shacham and is located in Newton, Massachusetts. For more information, please visit www.karyopharm.com.

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 PAK4 inhibitor, 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, 2014, which is on file with the Securities and Exchange Commission (SEC) as of March 13, 2015, 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.

CONDENSED CONSOLIDATED BALANCE SHEETS

(unaudited)

(in thousands, except share and per share amounts)

	March 31, 2015	December 31, 2014
Assets		
Current assets:		
Cash and cash equivalents	$ 51,453	$ 150,609
Short-term investments	194,956	55,115
Prepaid expenses and other current assets	2,910	2,027
Total current assets	249,319	207,751
Property and equipment, net	3,282	2,754
Long-term investments	38,539	8,658
Other assets	500	774
Restricted cash	400	400
Total assets	$ 292,040	$ 220,337
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 6,957	$ 6,288
Accrued expenses	7,417	5,825
Deferred rent	200	126
Other current liabilities	128	62
Total current liabilities	14,702	12,301
Deferred rent, net of current portion	1,813	1,242
Total liabilities	16,515	13,543

Stockholders' equity:		
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; 35,687,196 and 32,699,380 shares issued and outstanding at March 31, 2015 and December 31, 2014, respectively	4	3
Additional paid-in capital	439,914	345,166
Accumulated other comprehensive income (loss)	19	(29)
Accumulated deficit	(164,412)	(138,346)
Total stockholders' equity	275,525	206,794
Total liabilities and stockholders' equity	$ 292,040	$ 220,337

Karyopharm Therapeutics Inc.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS

(unaudited)

(in thousands, except share and per share amounts)

	Three Months Ended, March 31,	
	2015	**2014**
Contract and grant revenue	$ —	$ 171
Operating expenses:		
Research and development	20,751	10,979
General and administrative	5,399	2,904
Total operating expenses	26,150	13,883
Loss from operations	(26,150)	(13,712)
Other income (expense):		
Interest income	141	18
Other expense	(58)	—
Total other income (expense), net	83	18
Net loss	$ (26,067)	$ (13,694)
Net loss per share applicable to common stockholders—basic and diluted	$ (0.74)	$ (0.46)
Weighted-average number of common shares outstanding used in net loss per share applicable to common stockholders—basic and diluted	35,317,181	29,606,683

CONTACT: Justin Renz

(617) 658-0574

jrenz@karyopharm.com

Gina Nugent

(617) 460-3579

nugentcomm@aol.com

Source: Karyopharm Therapeutics

News Provided by Acquire Media