May 9, 2016

Karyopharm Reports First Quarter 2016 Financial Results and Highlights Recent Progress

Phase 2/3 SEAL Clinical Trial Initiated with Selinexor for Treatment of Liposarcoma

First-in-Human Study Initiated with Second-Generation SINE™ Compound KPT-8602 in Multiple Myeloma

Phase 2 Clinical Data in Multiple Myeloma Expected Mid-Year

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

NEWTON, Mass., May 09, 2016 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a clinical-stage pharmaceutical company, today reported financial results for the first quarter 2016 and commented on recent accomplishments and clinical development plans for its pipeline of several Selective Inhibitor of Nuclear Export (SINE™)-based therapeutics, including selinexor, its lead product candidate, as well as KPT-9274, its oral dual inhibitor of PAK4 and NAMPT.

"During the early part of 2016, Karyopharm continued to execute on its clinical strategy with the initiation of two clinical trials—a Phase 2/3 trial evaluating selinexor (KPT-330) in liposarcoma, and a first-in-human trial with its novel second-generation SINE compound, KPT-8602, in multiple myeloma," said Michael G. Kauffman, MD, PhD, Chief Executive Officer of Karyopharm. "We also highlighted the depth and breadth of our development pipeline with the presentation of preclinical data demonstrating encouraging activity with selinexor in combination with immune checkpoint inhibitors, a Bcl2 antagonist, and alkylating agents in oncology, KPT-8602 in multiple myeloma, KPT-350 in traumatic brain injury, and verdinexor (KPT-335) across a number of viral disease indications. Looking ahead, we expect to report top-line data from two of our ongoing studies, STORM and STOMP in relapsed/refractory multiple myeloma, during the second half of 2016."

Conference Call Information:

Karyopharm will host a conference call today, Monday, May 9, 2016, at 8:30 a.m. Eastern Time, to discuss the first quarter 2016 financial results, recent accomplishments, clinical developments and business plans. 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: 95849798. An audio recording 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.

Clinical Development Plans and Timelines:

- **Selinexor in hematologic malignancies**. Karyopharm is actively enrolling patients in several later phase clinical studies evaluating selinexor in hematologic malignancies, including patients with relapsed/refractory multiple myeloma (MM) (STORM study), older patients with relapsed/refractory acute myeloid leukemia (AML) (SOPRA study) and patients with relapsed/refractory diffuse large B-cell lymphoma (DLBCL) (SADAL study). Karyopharm expects data from the first 80 patients in the STORM study to be available in mid-2016, at which point, it will evaluate whether to expand the study. Karyopharm also expects to provide an interim analysis update from the SOPRA study in late 2016 with top-line data expected in mid-2017. Top-line data from the SADAL study is expected in early 2017.
- **Single-agent selinexor in solid tumors**. Karyopharm is currently conducting company-sponsored trials of single-agent selinexor in three solid tumor indications, including advanced unresectable dedifferentiated liposarcoma (SEAL study), heavily pretreated patients with gynecologic malignancies (SIGN study) and recurrent glioblastoma multiforme (KING study). The Phase 2/3 SEAL study, evaluating single-agent oral selinexor versus placebo, is supported by promising clinical data showing durable stable disease and improvement in progression free survival compared to previous chemotherapies. The primary endpoint of progression free survival (PFS) is acceptable to the FDA. Top-line data from the Phase 2 portion of this study are expected in mid-2017.
- **Selinexor combinations**. A number of investigator-sponsored and company-sponsored trials evaluating selinexor in combination with either chemotherapy or targeted agents in hematologic and solid tumor indications are currently ongoing or planned. In mid-2016, after meeting with the FDA, Karyopharm plans to initiate a Phase 2/3 study (SCORE study) evaluating the combination of selinexor, carfilzomib and dexamethasone in patients with refractory MM who were previously treated with a proteasome inhibitor and an immunomodulatory agent. In addition, Karyopharm expects to report top-line data from the Phase 1b portion of a Phase 1b/2 study (STOMP study) evaluating selinexor and dexamethasone in separate combinations with bortezomib, pomalidomide, or lenalidomide, for relapsed/refractory

MM in late 2016.

- **KPT-8602, second-generation SINE compound**. In January 2016, Karyopharm initiated a Phase 1/2 study of oral KPT-8602, a novel, second generation, SINE compound, in patients with relapsed/refractory MM. Top-line safety and tolerability data from the Phase 1 portion of this study are expected in late 2016.
- **KPT-9274, oral dual inhibitor of PAK4 and NAMPT**. In mid-2016, Karyopharm plans to initiate a Phase 1 clinical trial in patients with advanced solid malignancies (including sarcoma, colon and lung cancer) or non-Hodgkin's lymphoma (NHL). The primary endpoint of the study is expected to be the overall response rate measured at eight weeks.
- **Verdinexor (KPT-335).** In May 2015, Karyopharm began clinical testing of verdinexor in a randomized, double blind, placebo-controlled, dose-escalating Phase 1 clinical trial in healthy human volunteers in Australia. Verdinexor was found to be generally safe and well tolerated. Karyopharm plans to continue the clinical development of verdinexor as a potential treatment for influenza. Preclinical data provide strong support for other potential viral indications for verdinexor, including human immunodeficiency virus (HIV).
- **KPT-350.** KPT-350 is an investigational new drug application-ready oral compound with a preclinical data package supporting potential efficacy in a number of neurological, autoimmune and inflammatory conditions. Karyopharm plans to partner with a collaborator to undertake the clinical development and potential commercialization of KPT-350 in one or more mutually agreed indications. In March 2016, Karyopharm was awarded a grant of $225,000 from the National Institute of Allergy and Infectious Disease (NIAID) to advance development of KPT-350. The grant will be used to conduct further preclinical studies of KPT-350 for the treatment of systemic lupus erythematosus.

Scientific Presentations and Publications:

2016 American Association for Cancer Research (AACR) Annual Meeting, April 18-20, 2016 in New Orleans.

- Preclinical data demonstrating selinexor's ability to promote rapid tumor burden reduction in renal cell carcinoma models and its potential in combination with immunotherapy by priming the inflammatory and immune environment to maximize the effects of checkpoint inhibitors were presented. Previously, preclinical data showing selinexor's synergy with immune checkpoint inhibitors in melanoma and colon cancer models were presented.
- Preclinical data demonstrating that oral dosing daily x 5 each week with KPT-8602 lead to potent anti-leukemic activity, improved tolerability and prolonged survival compared with selinexor dosed twice weekly were also presented.

29th International Conference on Antiviral Research (ICAR), April 17-21, 2016 in La Jolla, California.
A number of viruses require XPO1 during their life cycle; XPO1 inhibition may inhibit viral replication in these cases. Preclinical data demonstrating the activity of verdinexor across a number of infectious disease indications including influenza, HIV, respiratory syncytial virus (RSV) and Venezuelan equine encephalitis virus (VEEV) were presented.

- Verdinexor showed potent, broad spectrum inhibition of multiple virus types known to utilize XPO1 as a single agent and synergistic reduction in viral titer in combination with standard of care in mouse models of influenza.
- SINE compounds demonstrated the ability to potently inhibit HIV replication and tumor growth with the potential for combined treatment of HIV- and AIDS-related cancers such as primary effusion lymphoma (PEL), a high-grade non-Hodgkin lymphoma typically affecting HIV-infected individuals.
- Prophylactic and therapeutic administration of verdinexor *in vitro* reduced viral replication across various RSV strains.
- Inhibition of VEEV, an arthropod-borne virus responsible for causing acute and fatal encephalitis, with verdinexor resulted in decreased viral replication and reduced viral titers.

2016 Annual Meeting of the American Academy of Neurology (AAN), April 15-21, 2016 in Vancouver, Canada.
Preclinical data demonstrating the activity of KPT-350, for the treatment of neuro-inflammatory disorders including traumatic brain injury (TBI), were presented:

- By inhibiting XPO1, KPT-350 demonstrated improved functional outcomes, tolerability, brain penetrance and therapeutic activity, including neuroprotective, anti-inflammatory and anti-epileptic effects in rodent models of TBI.

First Quarter 2016 Financial Results

Cash, cash equivalents and investments as of March 31, 2016, including restricted cash, totaled $187.1 million, compared to $210.0 million as of December 31, 2015.

For the quarter ended March 31, 2016, research and development expense was $21.8 million compared to $20.8 million for the quarter ended March 31, 2015. For the quarter ended March 31, 2016, general and administrative expense was $5.6 million compared to $5.4 million for the quarter ended March 31, 2015. The increase in research and development expenses resulted primarily from the increase in expenses related to the continued clinical development of selinexor.

Karyopharm reported a net loss of $27.1 million, or $0.75 per share, for the quarter ended March 31, 2016, compared to a net loss of $26.1 million, or $0.74 per share, for the quarter ended March 31, 2015. Net loss includes stock-based compensation expense of $5.2 million and $3.7 million for the quarters ended March 31, 2016 and March 31, 2015, 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 the middle of 2018, including advancing the STORM, SOPRA, SADAL and SEAL clinical studies to their next data inflection points. Karyopharm expects to end 2016 with at least $120 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 and related targets for the treatment of cancer and other major diseases. Karyopharm's SINE™ compounds function by binding with and inhibiting the nuclear export protein XPO1 (or CRM1). In addition to single-agent and combination activity against a variety of human cancers, SINE™ compounds have also shown biological activity in models of neurodegeneration, 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), KPT-8602, Karyopharm's next generation SINE compound, or KPT-9274, Karyopharm's first-in-class oral dual inhibitor of PAK4 and NAMPT, verdinexor (KPT-335), KPT-350, 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, 2015, which was filed with the Securities and Exchange Commission (SEC) on March 15, 2016, 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, 2016	December 31, 2015
Assets		
Current assets:		
Cash and cash equivalents	$ 42,188	$ 58,358

Short-term investments	94,228	117,275
Prepaid expenses and other current assets	1,726	1,967
Total current assets	138,142	177,600
Property and equipment, net	3,343	3,483
Long-term investments	50,165	33,878
Restricted cash	485	482
Total assets	$ 192,135	$ 215,443
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 4,109	$ 3,808
Accrued expenses	8,652	11,023
Deferred rent	263	206
Other current liabilities	198	95
Total current liabilities	13,222	15,132
Deferred rent, net of current portion	1,877	1,946
Total liabilities	15,099	17,078
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; 35,924,738 and 35,864,765 shares issued and outstanding at March 31, 2016 and December 31, 2015, respectively	4	4
Additional paid-in capital	460,524	455,170
Accumulated other comprehensive income (loss)	94	(282)
Accumulated deficit	(283,586)	(256,527)
Total stockholders' equity	177,036	198,365
Total liabilities and stockholders' equity	$ 192,135	$ 215,443

Karyopharm Therapeutics Inc.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
(unaudited)
(in thousands, except share and per share amounts)

	Three Months Ended, March 31,	
	2016	**2015**
Operating expenses:		
Research and development	$ 21,795	$ 20,751
General and administrative	5,554	5,399
Total operating expenses	27,349	26,150
Loss from operations	(27,349)	(26,150)
Other income (expense):		
Interest income	286	141
Other income (expense)	4	(58)
Total other income (expense), net	290	83
Net loss	$ (27,059)	$ (26,067)
Net loss per share—basic and diluted	$ (0.75)	$ (0.74)
Weighted-average number of common shares outstanding used in net loss per share—basic and diluted	35,878,502	35,317,181

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