Karyopharm Reports Second Quarter 2016 Financial Results and Highlights Recent Progress

Completed Enrollment in Phase 2b STORM Clinical Trial for Refractory Multiple Myeloma
On Track to Report Top-Line Data from STORM and STOMP Studies in Relapsed/Refractory Multiple Myeloma and Updated Data from SIGN Study in Gynecologic Malignancies
Conference Call Scheduled for Today at 8:30 a.m. ET

NEWTON, Mass., Aug. 04, 2016 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a clinical-stage pharmaceutical company, today reported financial results for the second quarter 2016 and commented on recent accomplishments and clinical development plans for its lead, novel, oral Selective Inhibitor of Nuclear Export (SINE™) compound selinexor (KPT-330), and other pipeline assets, including KPT-8602, its second-generation SINE™ compound, and KPT-9274, its oral, dual inhibitor of p21-activated kinase 4 (PAK4) and nicotinamide phosphoribosyltransferase (NAMPT).

"In the first half of 2016, we continued to drive rapid and meaningful progress across our development programs, including completion of enrollment in our Phase 2b STORM study in multiple myeloma (MM), the opening of a Phase 1b study arm evaluating selinexor in combination with the PD-1 inhibitor pembrolizumab in advanced solid tumors, and the advancement of KPT-9274 into the clinic," said Michael G. Kauffman, MD, PhD, Chief Executive Officer of Karyopharm. "At the 2016 European Hematology Association Annual Meeting, we presented exciting preliminary data from our Phase 1b STOMP study showing high response rates, along with durable activity and a favorable safety profile when selinexor is combined with standard-of-care agents in heavily pre-treated patients with MM."

Dr. Kauffman continued, "We remain on track for data readouts from several of our ongoing selinexor studies, including top-line results from the STORM and STOMP studies in relapsed/refractory MM, updated data from the SIGN study in gynecologic malignancies at the European Society of Medical Oncology 2016 Annual Meeting, and an interim analysis from the ongoing Phase 2 SOPRA study in relapsed/refractory acute myeloid leukemia (AML) in late 2016. For KPT-8602, our second generation SINE™ compound, we look forward to reporting top-line safety and tolerability results from the Phase 1 portion of the ongoing study in patients with relapsed/refractory MM in late 2016. All of these efforts bring us ever closer to our goal of providing novel, first-in-class medicines to patients with cancer and other major diseases. We look forward to keeping you updated on our progress."

Conference Call Information:

Karyopharm will host a conference call today, Thursday, August 4, 2016, at 8:30 a.m. Eastern Time, to discuss the second 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: 51644582. 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 has several ongoing clinical studies evaluating selinexor in hematologic malignancies, including selinexor in combination with low-dose dexamethasone in patients with MM (STORM study), in older patients with relapsed/refractory AML (SOPRA study), in patients with relapsed/refractory diffuse large B-cell lymphoma (DLBCL) (SADAL study) and in combination with backbone therapies in patients with MM (STOMP study).
    - Enrollment was completed in the Phase 2b STORM study. Of the 78 patients with measurable disease in the first cohort, 30 patients are penta-refractory. Karyopharm expects to report preliminary top-line data from the STORM study in September 2016, at which point the Company will announce the next steps in its MM development strategy.
    - The Company 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 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 U.S. Food and Drug Administration (FDA). Top-line data from the Phase 2 portion of this study are expected in mid-2017. The study was based on data recently published in the Journal of Clinical Oncology (Gounder et al., 2016).
    - Selinexor was recently granted Orphan Drug Designation from the FDA for use in the treatment of soft tissue sarcoma.
- Selinexor combinations. A number of investigator-sponsored and company-sponsored trials evaluating selinexor in combination with chemotherapeutic, targeted and immunotherapeutic agents in hematologic and solid tumor indications are currently ongoing or planned.
    - Karyopharm expects to report top-line data from the Phase 1b portion of a Phase 1b/2 study evaluating selinexor and dexamethasone in separate combinations with bortezomib, pomalidomide, or lenalidomide, for relapsed/refractory MM (STOMP study) in late 2016.
    - Karyopharm continues to assess the feasibility of the combination of selinexor, carfilzomib and dexamethasone in a Phase 2/3

study evaluating patients with refractory MM (SCORE study) who were previously treated with a proteasome inhibitor and an immunomodulatory agent.

- Patient dosing was recently initiated in a new arm of an ongoing investigator-sponsored Phase 1b clinical trial evaluating selinexor in combination with the PD-1 inhibitor pembrolizumab. Top-line data from this study, which is expected to enroll up to 470 patients across 13 treatment arms, are expected in early 2018.

- Patient dosing was also recently initiated in an investigator-sponsored Phase 1 clinical trial evaluating selinexor in combination with the oral proteasome inhibitor ixazomib and low dose dexamethasone in patients with relapsed/refractory MM.

- KPT-8602, second-generation SINE™ compound. In January 2016, Karyopharm initiated a Phase 1/2 clinical 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/NAMPT. In June 2016, Karyopharm initiated a Phase 1 clinical study in patients with advanced solid malignancies (including sarcoma, colon and lung cancer) or non-Hodgkin's lymphoma (NHL) whose disease has relapsed after standard therapy(s). The primary endpoints of this study are to determine the recommended Phase 2 dose and the maximum tolerated dose of KPT-9274 and to evaluate safety and tolerability. Top-line data from this study are expected in mid-2017.

- Verdinexor (KPT-335). In 2015, Karyopharm conducted a randomized, double blind, placebo-controlled, dose-escalating Phase 1 clinical trial in healthy human volunteers where 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). Verdinexor is also in development for the treatment of canine lymphoma, for which the safety and efficacy sections of a New Animal Drug Application have been submitted to the FDA's Center for Veterinary Medicine. If approved, verdinexor would be the first oral targeted therapy for the treatment of companion canine lymphoma.

- KPT-350. KPT-350 is an investigational new drug (IND) 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 addition to numerous grants awarded directly to our collaborators, in March 2016, Karyopharm was granted $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.

Second Quarter 2016 Financial Results

Cash, cash equivalents and investments as of June 30, 2016, including restricted cash, totaled $166.2 million, compared to $187.1 million as of March 31, 2016.

For the quarter ended June 30, 2016, research and development expense was $24.6 million compared to $27.0 million for the quarter ended June 30, 2015. For the quarter ended June 30, 2016, general and administrative expense was $6.0 million compared to $6.2 million for the quarter ended June 30, 2015. The decrease in research and development expenses resulted primarily from a decrease in costs associated with the completion of selinexor clinical trial supply manufacturing and toxicology studies in 2015.

Karyopharm reported a net loss of $30.2 million, or $0.84 per share, for the quarter ended June 30, 2016, compared to a net loss of $32.7 million, or $0.92 per share, for the quarter ended June 30, 2015. Net loss includes stock-based compensation expense of $6.4 million and $4.5 million for the quarters ended June 30, 2016 and June 30, 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). The Company's initial focus is on seeking regulatory approval and commercialization of its lead drug candidate, oral selinexor (KPT-330). To date, over 1,500 patients have been treated with selinexor and it is currently being evaluated in several mid- and later-stage clinical trials across multiple cancer indications, including acute myeloid leukemia (SOPRA), diffuse large B-cell lymphoma (SADAL), liposarcoma (SEAL) and in multiple myeloma in combination with low-dose dexamethasone (STORM) and backbone therapies (STOMP), among others. 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, which was founded by Dr. Sharon Shacham, currently has five investigational programs in clinical or preclinical development. 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 Quarterly Report on Form 10-Q for the quarter ended March 31, 2016, which was filed with the Securities and Exchange Commission (SEC) on May 9, 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)

	June 30, 2016	December 31, 2015
Assets		
Current assets:		
Cash and cash equivalents	$ 37,717	$ 58,358
Short-term investments	85,019	117,275
Prepaid expenses and other current assets	1,464	1,967
Total current assets	124,200	177,600
Property and equipment, net	3,170	3,483
Long-term investments	42,933	33,878
Restricted cash	483	482
Other assets	111	—
Total assets	$ 170,897	$ 215,443
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 4,063	$ 3,808
Accrued expenses	11,112	11,023
Deferred rent	269	206
Other current liabilities	79	95
Total current liabilities	15,523	15,132
Deferred rent, net of current portion	1,808	1,946
Total liabilities	17,331	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; 35,989,260 and 35,864,765 shares issued and outstanding at June 30, 2016 and December 31, 2015, respectively	4	4
Additional paid-in capital	467,168	455,170
Accumulated other comprehensive income (loss)	138	(282)
Accumulated deficit	(313,744)	(256,527)
Total stockholders' equity	153,566	198,365
Total liabilities and stockholders' equity	$ 170,897	$ 215,443

Karyopharm Therapeutics Inc.

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

	Three Months Ended, June 30,		Six Months Ended June 30,	
	2016	2015	2016	2015
Contract and grant revenue	$ 59	$ 150	$ 59	$ 150
Operating expenses:				

Research and development	24,579	27,006	46,374	47,757
General and administrative	5,956	6,157	11,510	11,556
Total operating expenses	30,535	33,163	57,884	59,313
Loss from operations	(30,476)	(33,013)	(57,825)	(59,163)
Other income:				
Interest income	329	267	615	408
Other income (expense)	(11)	51	(7)	(7)
Total other income, net	318	318	608	401
Net loss	$ (30,158)	$ (32,695)	$ (57,217)	$ (58,762)
Net loss per share—basic and diluted	$ (0.84)	$ (0.92)	$ (1.59)	$ (1.65)
Weighted-average number of common shares outstanding used in net loss per share—basic and diluted	35,956,470	35,697,012	35,917,486	35,508,146

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