November 9, 2015

Karyopharm Reports Third Quarter 2015 Financial Results and Highlights Recent Progress

- Robust Recruitment and New Study Initiations Continue -

- Additional Encouraging Data on Selinexor in Combination with Other Active Agents to be Presented at ASH -

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

NEWTON, Mass., Nov. 9, 2015 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a clinical-stage pharmaceutical company, today reported financial results for the third quarter 2015 and commented on recent accomplishments and clinical development plans for its pipeline of SINE™ based therapeutics including selinexor, its lead product candidate.

"This has been a very busy period for Karyopharm with active enrollment across our ongoing selinexor clinical studies and new studies, primarily in combination with other anticancer agents, continuing to come on-line," said Michael G. Kauffman, MD, PhD, Chief Executive Officer of Karyopharm. "We look forward to sharing additional data on our pipeline of first-in-class oncology therapeutics at the upcoming American Society of Hematology 2015 annual meeting, including selinexor activity in combination regimens across hematologic malignancies."

Conference Call Information:

Karyopharm will host a conference call today, Monday, November 9, 2015, at 8:30 a.m. Eastern Time, to discuss the third quarter 2015 financial results, recent accomplishments and clinical developments 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: 69164993. 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:

- Based on striking preclinical synergy in animal models of myeloma, Karyopharm initiated a multi-center, Phase 1b/2 clinical study of selinexor called STOMP ("**S**elinexor and Backbone **T**reatments **o**f Multiple **M**yeloma **P**atients") with support from Myeloma Canada. In this multi-arm study, Karyopharm is evaluating the combination of selinexor and low dose dexamethasone with backbone therapies bortezomib, pomalidomide or lenalidomide in patients with previously treated multiple myeloma. Approximately 220 patients with multiple myeloma will be enrolled in this study with preliminary top-line data anticipated in 2017. Selinexor and low dose dexamethasone are already being combined with carfilzomib in an Investigator-sponsored trial (IST), where promising preliminary data were presented at the ASH 2014 annual meeting and will be updated with additional patient data at the ASH 2015 annual meeting.
- Karyopharm is actively enrolling patients in four later phase clinical studies evaluating single-agent selinexor: one in older patients with relapsed/refractory acute myeloid leukemia (SOPRA study), the second in patients with relapsed/refractory diffuse large B-cell lymphoma (SADAL study), the third in patients with multiple myeloma (STORM study) and the fourth in patients with Richter's transformation (SIRRT study). Interim data are expected from the SOPRA and STORM studies in the middle of 2016. Preliminary top-line data from the SOPRA and SADAL studies are anticipated in the fourth quarter of 2016.
- Karyopharm is currently conducting company-sponsored trials of single-agent selinexor in three solid tumor indications including heavily pretreated patients with gynecologic malignancies (SIGN study), recurrent glioblastoma multiforme (KING study) and hormone-refractory prostate cancer (SHIP study). Based on promising data observed in a Phase 1 study, a randomized, blinded Phase 2/3 trial of selinexor versus placebo in liposarcoma (SEAL study) is planned to commence in the fourth quarter of 2015.
- In addition, a number of ISTs or company-sponsored trials evaluating the potential of selinexor in combination with either chemotherapy or targeted agents are currently ongoing or planned.
- Based on promising preclinical data, Karyopharm filed an Investigational New Drug (IND) application with the Food and Drug Administration (FDA) for KPT-8602 and, pending review, plans to initiate a clinical study in multiple myeloma in early 2016.
- Karyopharm also plans to file an IND for its first-in-class, oral PAK4 Allosteric Modulator, KPT-9274, and initiate clinical development in patients with heavily pretreated solid tumors or lymphoma in the first half of 2016.

Scientific Presentations and Publications:

- Five oral presentations and twelve poster presentations describing Karyopharm's pipeline of first-in-class oncology therapeutics were accepted for presentation at the upcoming 57th Annual American Society of Hematology (ASH) 2015 meeting being held December 5 - 8, 2015 in Orlando, Florida. The accepted abstracts include:
    - Clinical and preclinical data demonstrating the promising activity of selinexor (KPT-330), Karyopharm's most advanced, novel, oral Selective Inhibitor of Nuclear Export/SINE compound, in combination with other anti-cancer agents;
    - Preclinical data on KPT-8602, a second generation SINE compound in early-stage development with the potential for distinct pharmaceutical characteristics; and
    - Preclinical data on KPT-9274, a first-in-class oral PAK4 Allosteric Modulator.
- Preclinical data demonstrating the anti-tumor benefits of combining selinexor with immunotherapy in aggressive melanoma models were presented at the Society for Immunotherapy Cancer (SITC) 2015 Annual Meeting held November 4 - 8, 2015 in National Harbor, Maryland. In an oral presentation entitled "Selinexor, a Selective Inhibitor of Nuclear Export (SINE), enhanced activity in combination with PD-1/PD-L1 blockade in syngeneic murine models of colon cancer and melanoma" Karyopharm researchers and collaborators at The Ohio State University demonstrated that the combination of selinexor with PD-1 or PDL-1 immune checkpoint inhibitors exerts considerable anti-tumor and immune-stimulating activity in an aggressive murine melanoma model.
- Data demonstrating the beneficial pharmacological effects of SINE-based compounds in neurodegenerative models, including Amyotrophic Lateral Sclerosis (ALS), were recently published in the journal *Nature* and presented at the American Neurological Association (ANA) and the Society for Neuroscience (SfN) meetings. These data confirm that nuclear transport is disrupted by a common gene mutation found in ALS and describe the neuroprotective effects of SINE compounds, similar to the neuroprotective effects previously observed in Multiple Sclerosis (MS). Karyopharm's SINE compound research efforts in ALS are being supported entirely by collaborator grant funding, with MS research supported by the National Multiple Sclerosis Society. Given the tremendous unmet need for new treatments for ALS and MS, Karyopharm is advancing its oral SINE compound KPT-350 for these indications.

Third Quarter September 30, 2015 Financial Results

Cash, cash equivalents and investments as of September 30, 2015, including restricted cash, totaled $230.2 million, compared to $256.0 million as of June 30, 2015.

For the quarter ended September 30, 2015, research and development expense was $25.9 million compared to $16.0 million for the quarter ended September 30, 2014. For the quarter ended September 30, 2015, general and administrative expense was $4.8 million compared to $3.8 million for the quarter ended September 30, 2014. The increase in research and development expense 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 $30.4 million, or $0.85 per share, for the quarter ended September 30, 2015, compared to a net loss of $19.7 million, or $0.61 per share, for the quarter ended September 30, 2014. Net loss includes stock-based compensation expense of $3.5 million and $2.9 million for the quarters ended September 30, 2015 and September 30, 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 the four later-stage 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 with and inhibiting the nuclear export protein XPO1 (or CRM1). Karyopharm's lead drug candidate, selinexor (KPT-330), is a first-in-class, oral Selective Inhibitor of Nuclear Export / SINE compound. Selinexor functions by binding with and inhibiting the nuclear export protein XPO1 (also called CRM1), leading to the accumulation of tumor suppressor proteins in the cell nucleus. In 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 http://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 PAK4 Allosteric Modulator, 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 September 30, 2015, which is on file with the Securities and Exchange Commission (SEC) as of November 9, 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)

	September 30, 2015	December 31, 2014
Assets		
Current assets:		
Cash and cash equivalents	$43,499	$150,609
Short-term investments	151,738	55,115
Prepaid expenses and other current assets	3,107	2,027
Total current assets	198,344	207,751
Property and equipment, net	3,660	2,754
Long-term investments	34,430	8,658
Other assets	52	774
Restricted cash	485	400
Total assets	$236,971	$220,337
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$4,568	$6,288
Accrued expenses	9,469	5,825
Deferred rent	200	126
Other current liabilities	203	62

Total current liabilities	14,440	12,301
Deferred rent, net of current portion	1,939	1,242
Total liabilities	16,379	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; 35,711,950 and 32,699,380 shares issued and outstanding at September 30, 2015 and December 31, 2014, respectively	4	3
Additional paid-in capital	448,095	345,166
Accumulated other comprehensive loss	(26)	(29)
Accumulated deficit	(227,481)	(138,346)
Total stockholders' equity	220,592	206,794
Total liabilities and stockholders' equity	$236,971	$220,337

Karyopharm Therapeutics Inc.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS

(unaudited)

(in thousands, except share and per share amounts)

	Three Months Ended, September 30,		Nine Months Ended, September 30,	
	2015	2014	2015	2014
Contract and grant revenue	$75	$21	$225	$214
Operating expenses:				
Research and development	25,923	15,951	73,680	40,089
General and administrative	4,762	3,814	16,318	10,028
Total operating expenses	30,685	19,765	89,998	50,117
Loss from operations	(30,610)	(19,744)	(89,773)	(49,903)
Other income (expense):				
Interest income	239	20	647	54
Other expense	(2)	—	(9)	—
Total other income (expense), net	237	20	638	54
Net loss	$ (30,373)	$ (19,724)	$ (89,135)	$ (49,849)
Net loss per share—basic and diluted	$ (0.85)	$ (0.61)	$ (2.51)	$ (1.63)
Weighted-average number of common shares outstanding used in net loss per share—basic and diluted	35,708,739	32,558,646	35,575,745	30,619,074

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