Karyopharm Reports Fourth Quarter and Full Year 2015 Financial Results and Highlights Recent Progress

Company Continues to Expand its Leadership in SINETM-based Therapies with Selinexor Clinical Advancement and Presentations of Encouraging Data for Earlier Stage Pipeline Programs
Cash Expected to Fund Current R&D Plans into 2018
Several Data Readouts Expected over the Next 18 Months
Conference Call Scheduled for Today at 8:30 a.m. ET

NEWTON, Mass., March 14, 2016 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a clinical-stage pharmaceutical company, today reported financial results for the fourth quarter and full year 2015 and commented on recent accomplishments and clinical development plans for its pipeline of several SINE-based therapeutics including selinexor, its lead product candidate.

"During 2015, we continued to expand our leadership position in the development of oral SINE-based oncology and non-oncology therapies with clinical advancement of selinexor across a number of hematologic and solid tumor indications and the presentation of encouraging data on our earlier-stage pipeline programs," said Michael G. Kauffman, MD, PhD, Chief Executive Officer of Karyopharm. "Our broad and aggressive oncology clinical development program continues in 2016 with both company- and investigator-sponsored trials underway or planned to evaluate selinexor both as a single-agent and in combination with other existing and emerging oncology therapies. In addition, we made important progress with our non-oncology focused SINE compounds in a number of disease areas in which XPO1 is critically involved. We look forward to several potentially value-creating data readouts over the next 18 months."

Conference Call Information:

Karyopharm will host a conference call today, Monday, March 14, 2016, at 8:30 a.m. Eastern Time, to discuss the fourth quarter and full-year 2015 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: 59463471. 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:

- Selinexor in hematologic malignancies. Karyopharm is actively enrolling patients in several later phase clinical studies evaluating single-agent 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. Based on discussions with the U.S. Food and Drug Administration (FDA), Karyopharm amended the protocol for the ongoing SADAL study to remove dexamethasone from the regimen and to evaluate selinexor as a single-agent in this patient population. The study will continue to compare 60 mg and 100mg twice weekly doses of selinexor with 100 patients per arm and at least 50% of patients with GCB-type DLBCL. Based on these amendments, the company expects to report top-line data from this study 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). Karyopharm is planning to present updated data from the SIGN and KING studies at an appropriate medical meeting in mid-2016. The Phase 2/3 SEAL study, evaluating single-agent oral selinexor versus placebo, was initiated earlier this year based on promising clinical data presented at the American Society of Clinical Oncology (ASCO) 2015 Annual Meeting in which durable stable disease and improvement in progression free survival compared to previous chemotherapies were observed. 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, 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 1 portion of a Phase 1b/2 study (STOMP study) evaluating selinexor in combination with commonly used treatments, including 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. Data from preclinical studies presented at the 2015 American Society of Hematology (ASH) Annual Meeting demonstrated single-agent anti-MM activity of KPT-8602 along with the potential for higher and/or more frequent dosing compared with selinexor. Top-line safety and tolerability data from this Phase 1/2 study are expected in late 2016.
- KPT-9274, oral dual inhibitor of PAK4 and NAMPT. In mid-2016, Karyopharm plans to initiate clinical development in patients with heavily pretreated solid tumors or lymphoma for its oral dual inhibitor of PAK4 and NAMPT, KPT-9274.
- 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 is preparing to advance verdinexor for certain viral indications with an initial focus on influenza. Preclinical data provide strong support for other potential 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. We plan to partner with a collaborator to undertake the clinical development and potential commercialization of KPT-350 in one or more mutually agreed indications.

Scientific Presentations and Publications:

- Positive clinical data demonstrating the activity and tolerability of selinexor across multiple hematologic malignancies including MM and AML were presented at the 2015 American Society of Hematology (ASH) Annual Meeting held in December 2015 in Orlando,

Florida.

- Updated data from an ongoing investigator-sponsored trial of selinexor in combination with carfilzomib and dexamethasone in refractory MM were presented by Andrzej Jakubowiak, MD, PhD, from The University of Chicago. The data demonstrated encouraging efficacy of selinexor in combination with carfilzomib and dexamethasone in heavily pretreated patients with highly refractory MM, including patients whose disease is refractory to previous carfilzomib-based combinations, suggesting the potential of this combination to overcome carfilzomib resistance. Based on these data, Karyopharm expects to initiate a Phase 2/3 clinical study (SCORE study) in mid-2016.
- Karyopharm researchers and collaborators also presented preclinical data demonstrating the potential of selinexor to synergize with proteasome inhibitors to overcome drug resistance in MM.
- Karyopharm presented data demonstrating the activity and tolerability of selinexor in combination with standard of care agents in the AML setting, including data from a Phase 2 trial investigating the efficacy and tolerability of selinexor in combination with arabinoside cytosine (Ara-C) and idarubicin in "fit" patients with relapsed and/or refractory AML. Karyopharm also presented data from a Phase 1 study determining the safety and efficacy of selinexor in combination with fludarabine and cytarabine in pediatric patients with relapsed and/or refractory acute leukemia.
- Karyopharm researchers presented data establishing 60mg as the most appropriate selinexor dose for both efficacy and tolerability across many hematologic and solid cancers. These data support the selinexor first-in-human Phase 1 clinical trial solid tumor data published in *Journal of Clinical Oncology* in March 2016, which recommended a dose of 60mg given twice a week as the Phase 2 dose. This recommendation was based on superior patient tolerability, longer duration of therapy, and no demonstrable improvement in radiologic response or disease stabilization compared with higher doses.

Fourth Quarter and Year Ended December 31, 2015 Financial Results

Cash, cash equivalents and investments as of December 31, 2015, including restricted cash, totaled $210.0 million, compared to $214.8 million as of December 31, 2014.

For the year ended December 31, 2015, research and development expense was $97.7 million compared to $60.1 million for the year ended December 31, 2014. For the year ended December 31, 2015, general and administrative expense was $21.6 million compared to $15.9 million for the year ended December 31, 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 an increase in personnel costs, including stock-based compensation expense.

Karyopharm reported a net loss of $118.2 million, or $3.32 per share, for the year ended December 31, 2015, compared to a net loss of $75.8 million, or $2.43 per share, for the year ended December 31, 2014. Net loss includes stock-based compensation expense of $17.1 million and $14.2 million for the years ended December 31, 2015 and 2014, respectively.

For the quarter ended December 31, 2015, research and development expense was $24.1 million compared to $20.0 million for the quarter ended December 31, 2014. For the quarter ended December 31, 2015, general and administrative expense was $5.3 million compared to $5.9 million for the quarter ended December 31, 2014. 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 $29.0 million, or $0.81 per share, for the quarter ended December 31, 2015, compared to a net loss of $25.9 million, or $0.79 per share, for the quarter ended December 31, 2014. Net loss includes stock-based compensation expense of $5.4 million and $4.6 million for the quarters ended December 31, 2015 and December 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 the middle of 2018, including advancing the four later-stage 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) , leading to the accumulation of tumor suppressor proteins in the cell nucleus. Karyopharm's lead drug candidate, selinexor (KPT-330), is a first-in-class, oral Selective Inhibitor of Nuclear Export / SINE compound. In addition to single-agent and combination activity against a variety of different human cancers, SINE compounds have also shown biological activity in models of inflammation, autoimmune disease, neurological disorders, 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, 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 was filed with the Securities and Exchange Commission (SEC) on 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.
Consolidated Balance Sheets
(unaudited)
(in thousands, except share and per share amounts)

	December 31, 2015	December 31, 2014
ASSETS		
Current assets:		
Cash and cash equivalents	$ 58,358	$ 150,609
Short-term investments	117,275	55,115
Prepaid expenses and other current assets	1,967	2,027
Total current assets	177,600	207,751
Property and equipment, net	3,483	2,754
Long-term investments	33,878	8,658
Other assets	—	774
Restricted cash	482	400
Total assets	$ 215,443	$ 220,337
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 3,808	$ 6,288
Accrued expenses	11,023	5,825
Deferred rent	206	126
Other current liabilities	95	62
Total current liabilities	15,132	12,301
Deferred rent, net of current portion	1,946	1,242
Total liabilities	17,078	13,543
Commitments and contingencies		
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; 35,864,765 and 32,699,380 shares issued and outstanding at December 31, 2015 and 2014, respectively	4	3
Additional paid-in capital	455,170	345,166
Accumulated other comprehensive loss	(282)	(29)
Accumulated deficit	(256,527)	(138,346)
Total stockholders' equity	198,365	206,794
Total liabilities and stockholders' equity	$ 215,443	$ 220,337

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

	For the Quarter Ended, December 31,	For the Year Ended December 31,

	2015	2014	2015	2014
Contract and grant revenue	$ 25	$ 16	$ 250	$ 229
Operating expenses:				
Research and development	24,064	20,038	97,744	60,127
General and administrative	5,264	5,920	21,582	15,948
Total operating expenses	29,328	25,958	119,326	76,075
Loss from operations	(29,303)	(25,942)	(119,076)	(75,846)
Other income (expense):				
Interest income	250	42	897	97
Other expense	7	(28)	(2)	(28)
Total other income (expense), net	257	14	895	69
Net loss	$ (29,046)	$ (25,928)	$ (118,181)	$ (75,777)
Net loss per share—basic and diluted	$ (0.81)	$ (0.79)	$ (3.32)	$ (2.43)
Weighted-average number of common shares outstanding used in net loss per share—basic and diluted	35,749,362	32,668,705	35,619,506	31,135,694

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https://investors.karyopharm.com/2016-03-14-Karyopharm-Reports-Fourth-Quarter-and-Full-Year-2015-Financial-Results-and-Highlights-Recent-Progress