Karyopharm Reports Third Quarter 2014 Financial Results and Highlights Recent Progress

- Reported Positive Phase 1 Clinical Data Demonstrating Early Signs of Activity for Selinexor in Several Solid Tumor Settings -
- Initiated SIRTT, an Additional Registration-Directed Trial for Relapsed/Refractory Richter's Transformation -
- Provides Preview on Several Presentations and Posters at Upcoming ASH Meeting -
- Conference Call Scheduled for Today at 8:30 a.m. ET -

NEWTON, Mass., Nov. 10, 2014 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a clinical-stage pharmaceutical company, today reported financial results for the third quarter ended September 30, 2014 and also commented on recent accomplishments and clinical development plans.

"We are excited by the progress we made this quarter expanding our oncology development programs, including our lead product candidate, Selinexor, a first-in-class, oral Selective Inhibitor of Nuclear Export / SINE™ compound, as well as building our leadership team and global footprint to pursue an aggressive commercialization strategy," said Dr. Michael Kauffman, CEO of Karyopharm. "We now have two registration-directed Selinexor clinical studies ongoing, one in acute myeloid leukemia and the other in Richter's Transformation, which represent significant advancements in our accelerated development program for severe hematologic indications with high unmet medical need."

Conference Call Information:

To access the conference call, please dial (855) 437-4406 or (484) 756-4292 (international) at least five minutes prior to the start time and refer to conference ID 24693830. A live audio webcast of the call will be available on the Investors & Media section of the company's website, investors.karyopharm.com/events.cfm. An archived audio webcast will be available on the company's website approximately two hours after the event.

Recent Corporate Accomplishments

- Initiated an Additional Registration-Directed Trial with Selinexor (KPT-330):
    - The company initiated a Phase 2 study called SIRRT, or Selinexor in Relapsed and/or refractory Richter's Transformation. Richter's Transformation is a rare condition (approximately 1,500 patients annually in the United States) in which chronic lymphocytic leukemia (CLL) changes into a fast-growing type of lymphoma. There are currently no FDA-approved agents for this disease. This single-arm, open-label, multi-center study of Selinexor will enroll approximately 50 patients in approximately 35 sites worldwide. This second-line study will evaluate the safety and efficacy of Selinexor given orally to patients with Richter's Transformation whose disease has relapsed after, or is refractory to, chemotherapy. Enrolled patients will receive Selinexor (60 mg/m2) twice weekly each week. Overall response rate is the primary endpoint of this study.

- Scientific Updates at Major Conferences:
    - Clinical and preclinical data for Selinexor will be presented at the upcoming 56th American Society of Hematology (ASH) Annual Meeting being held from December 6-9, 2014 in San Francisco. This growing body of data for Selinexor in hematologic malignancies includes promising Selinexor clinical data in patients with heavily pretreated, relapsed and refractory aggressive non-Hodgkin's lymphoma and additional preclinical and clinical data demonstrating synergistic activity of Selinexor when combined with dexamethasone in multiple myeloma (MM). Data to be presented include:
        - An oral presentation on Selinexor in non-Hodgkin's lymphoma titled"The Oral Selective Inhibitor of Nuclear Export (SINE) Selinexor (KPT-330) Demonstrates Broad and Durable Clinical Activity in Relapsed / Refractory Non-Hodgkin's Lymphoma (NHL)," to be presented on Monday, December 8, 2014 at 11:45 AM PT. Highlights of the topline abstract data include: Overall response rate (partial response or better) of 40% and disease control rate (stable disease or better) of 80% in 10 patients with relapsed and/or refractory aggressive non-Hodgkin's lymphoma treated with single-agent Selinexor dosed twice weekly at ≥60mg/m2 (equivalent to approximately 100mg doses). A 33% response rate was observed in 27 patients dosed at 35-50mg/m2 (equivalent to approximately 60-85mg doses), along with a 25% response rate at lower doses. These data are current through August 5, 2014, and will be updated in detail at ASH.
        - A poster presentation titled, "Selinexor Demonstrates Marked Synergy with Dexamethasone (Sel-Dex) in Preclinical Models and in Patients with Heavily Pretreated Refractory Multiple Myeloma (MM)," to be presented on Monday, December 8, 2014, 6:00 PM - 8:00 PM PT. Highlights of the topline abstract data include: Overall response rate (partial response or better) of 60% and clinical benefit rate (minimal response or better) of 80% in 10 patients with heavily pretreated and refractory multiple myeloma treated with Selinexor in combination with low-dose dexamethasone, each dosed twice weekly at 45mg/m2 and 20mg, respectively. These data are current through August 5, 2014, and will be updated in detail at ASH. This 45mg/m2 dose of Selinexor with dexamethasone will be taken forward in additional studies in patients with heavily pretreated MM.
        - Additional posters detailing preclinical data from Karyopharm's PAK4 inhibitor program, as well as preclinical data of Selinexor in acute myeloid leukemia (AML), will also be presented. In addition, investigators and collaborators will be presenting data for Selinexor in combination with other therapies.

- Karyopharm presented clinical data from an ongoing Phase 1 clinical study of Selinexor in a diverse set of heavily pretreated solid tumors at the 2014 Congress of the European Society for Medical Oncology (ESMO), held September 26-30, 2014 in Madrid, Spain. Single-agent Selinexor demonstrated a 60% disease control rate with maximum prostate-specific antigen (PSA) reduction ranging from 27% to 60% and duration of treatment up to 502 days. Selinexor also demonstrated early signs of clinical activity in other solid tumor indications including head and neck squamous cell carcinoma and ovarian cancer. Selinexor was shown to have manageable and predictable side effects, primarily nausea, fatigue and anorexia, which improve over time on treatment.

- Based on the favorable safety profile to date and encouraging efficacy data, Karyopharm has initiated Phase 2 trials with Selinexor

in multiple solid tumor indications.

- Strengthened Leadership Team and Expanded Global Footprint:
  - Expanded Karyopharm's global presence with a wholly-owned European subsidiary and appointed Ran Frenkel, an international executive with significant experience managing European clinical operations and regulatory affairs within the biopharmaceutical industry, as Executive Vice President, Worldwide Development Operations in October 2014. The new subsidiary, Karyopharm Europe GmbH, will have its headquarters in Munich, Germany and will provide the corporate structure necessary to support Karyopharm's expanded clinical and regulatory activities in Europe.
  - Appointed Scott Garland, Senior Vice President and Chief Commercial Officer at Relypsa, to the company's board of directors in November 2014. Mr. Garland brings nearly 25 years of oncology commercial leadership to Karyopharm, including experience at Genentech, Amgen, Merck and Exelixis. Mr. Garland's skills and experience in leading oncology-focused sales and marketing organizations within the biopharmaceutical industry will be valuable to Karyopharm as the company advances the Selinexor development program toward commercialization.
  - Appointed Justin Renz as Executive Vice President and Chief Financial Officer in August 2014. With extensive experience in the biopharmaceutical industry, Mr. Renz most recently served as Executive Vice President and Chief Financial Officer at Zalicus Inc. (formerly CombinatoRx, Inc.), where he led core business and finance functions, oversaw multiple rounds of equity and debt financing, led the company's asset monetization strategy and most recently, was instrumental in the reverse merger and sale of Zalicus to EPIRUS Biopharmaceuticals Inc. Prior to Zalicus, Mr. Renz served in senior finance and accounting roles at Serono, Inc. and Coley Pharmaceutical Group, Inc. Earlier in his career, Mr. Renz held increasingly senior finance positions at ArQule, Inc. and Millipore Corporation.

Regulatory/Intellectual Property Updates:

- Received U.S. Food and Drug Administration (FDA) and European Medicines Agency (EMA) orphan drug status for Selinexor in both AML and diffuse large B-Cell lymphoma (DLBCL).
- Received U.S. patent allowance covering composition of matter for Selinexor. Once issued, this patent will provide patent protection for Selinexor and pharmaceutical compositions comprising Selinexor through the middle of 2032.

Clinical Development Plans:

- Karyopharm is actively enrolling patients in a registration-directed, randomized Phase 2 study of Selinexor in approximately 150 patients 60 years of age or older with relapsed or refractory AML who are ineligible for intensive chemotherapy and/or transplantation. The first patient in the Selinexor in Older Patients with Relapsed/Refractory AML (SOPRA) study was dosed in June 2014.
- Karyopharm is actively enrolling patients in a registration-directed Phase 2 study of Selinexor in approximately 50 patients with relapsed and/or refractory Richter's Transformation. The Selinexor in Patients with Refractory and/or Relapsed Richter's Transformation (SIRRT) study was initiated in November 2014.
- Karyopharm plans to initiate the SADAL study, or Selinexor and Dexamethasone in Aggressive Lymphoma, a registration-directed, randomized Phase 2 study of Selinexor in patients with relapsed and/or refractory DLBCL in the fourth quarter of 2014.
- Karyopharm plans to meet with the FDA during the fourth quarter of 2014 to discuss the criteria and study design for potential accelerated approval of Selinexor in patients with relapsed/refractory MM.
- Karyopharm is actively enrolling patients in four Phase 2 solid tumor studies evaluating Selinexor in gynecologic malignancies (SINE Study), glioblastoma multiforme (KING Study), metastatic prostate cancer (SHIP Study) and squamous head and neck, lung and esophageal cancers (STARRS Study) with the goal of reporting interim data at an oncology meeting in mid-2015.
- A number of investigator-sponsored (ISTs) or company-sponsored clinical studies evaluating the potential of Selinexor in combination with other therapies are currently ongoing or planned, including AML in combination with low dose AraC, AML in combination with idarubicin and AraC, MM in combination with carfilzomib, CLL in combination with ibrutinib, MM in combination with pomalidomide and dexamethasone, and MM in combination with liposomal doxorubicin (Doxil®).

Third Quarter 2014 Financial Results

Cash and cash equivalents as of September 30, 2014, totaled $227.1 million compared to $156.0 million as of December 31, 2013.

For the quarter ended September 30, 2014, research and development expense was $16.0 million compared to $7.7 million for the same period in the previous year. For the quarter ended September 30, 2014, general and administrative expense was $3.8 million compared to $1.6 million for the same period in the previous year. The increase in research and development expenses resulted primarily from the increase in expenses related to the significantly expanded clinical development activities for our lead drug candidate, Selinexor (KPT-330). The increase in general and administrative expense resulted primarily from the costs of being a public company and an increase in stock-based compensation expense.

Karyopharm reported a net loss of $19.7 million, or $0.61 per share, for the quarter ended September 30, 2014, compared to a net loss of $9.3 million, or $3.66 per share, for the same period in the previous year. Net loss includes stock-based compensation expense of $2.9 million and $1.3 million for the quarters ended September 30, 2014 and 2013, respectively.

For the nine months ended September 30, 2014, research and development expense was $40.1 million compared to $18.8 million for the same period in the previous year. For the nine months ended September 30, 2014, general and administrative expense was $10.0 million compared to $3.4 million for the same period in the previous year. 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 $49.8 million, or $1.63 per share, for the nine months ended September 30, 2014, compared to a net loss of $21.8 million, or $9.11 per share, for the same period in the previous year. Net loss includes stock-based compensation expense of $9.6 million and $1.8 million for the nine months ended September 30, 2014 and 2013, respectively.

Financial Guidance

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 second half of 2017, including moving our two registration-directed clinical studies to their next data inflection points. Karyopharm expects to end 2014 with greater than $200 million in cash and cash equivalents.

About Selinexor

Selinexor (KPT-330) is a first-in-class, oral Selective Inhibitor of Nuclear Export / SINE™ compound. Selinexor functions by inhibiting the nuclear export protein XPO1 (also called CRM1), leading to the accumulation of tumor suppressor proteins in the cell nucleus, which subsequently reinitiates and amplifies their tumor suppressor function. This is believed to lead to the selective induction of apoptosis in cancer cells, while largely sparing normal cells. Over 450 patients have been treated with Selinexor in Phase 1 and Phase 2 clinical trials in advanced hematologic malignancies and solid tumors. Karyopharm has initiated two registration-directed clinical trials of Selinexor, one in older patients with acute myeloid leukemia and the other in patients with Richter's Transformation. Two additional registration-directed clinical trials in hematological indications, one in patients with diffuse large B-cell lymphoma (DLBCL) and the other in patients with multiple myeloma, are expected to begin enrollment during the fourth quarter of 2014 and first half of 2015, respectively. Additional Phase 1 and Phase 2 studies are ongoing or currently planned, including multiple investigator-sponsored studies of Selinexor in combination with one or more approved therapies. The latest clinical trial information for Selinexor is available at www.clinicaltrials.gov.

About Karyopharm Therapeutics

Karyopharm Therapeutics Inc. (Nasdaq:KPTI) is a clinical-stage pharmaceutical company focused on the discovery and development of novel 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). SINE™ compounds have also shown biological activity in models of 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, 2013, which is on file with the Securities and Exchange Commission (SEC), 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.
Unaudited Selected Consolidated Balance Sheet Information
(in thousands)

	September 30, 2014	December 31, 2013
Cash and cash equivalents	$227,131	$155,974
Prepaid expenses and other current assets	4,215	1,982
Property and equipment, net	2,734	240
Other assets	1,075	30
Total assets	$235,155	$158,226
Accounts payable and accrued expenses	$5,751	$2,908
Other liabilities	1,600	384
Stockholders' equity	227,804	154,934
Total liabilities and stockholders' equity	$235,155	$158,226

Karyopharm Therapeutics Inc.
Unaudited Condensed Consolidated Statement of Operations
(in thousands, except share and per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2014	2013	2014	2013
Revenue:				

Contract and grant revenue	$21	$ --	$214	$366
Operating expenses:				
Research and development	15,951	7,738	40,089	18,763
General and administrative	3,814	1,583	10,028	3,405
Total operating expenses	19,765	9,321	50,117	22,168
Loss from operations	(19,744)	(9,321)	(49,903)	(21,802)
Interest income	20	--	54	1
Net loss	($19,724)	($9,321)	($49,849)	($21,801)
Net loss per share applicable to common stockholders-basic and diluted	($0.61)	($3.66)	($1.63)	($9.11)
Weighted-average number of common shares used in net loss per share applicable to common stockholders-basic and diluted	32,558,646	2,544,587	30,619,074	2,392,589

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