Karyopharm Presents Clinical Data for Selinexor (KPT-330) in Patients With Heavily Pretreated Gynecological Cancers and Asian Patients With Advanced Malignancies at 2015 ASCO Annual Meeting

NEWTON, Mass., May 30, 2015 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a clinical-stage pharmaceutical company, today announced the presentation of positive clinical data for its lead product candidate, Selinexor (KPT-330), a first-in-class, oral Selective Inhibitor of Nuclear Export / SINE™ compound, at the 2015 American Society of Clinical Oncology (ASCO) Annual Meeting. In an ongoing Phase 2 clinical trial evaluating the activity of single-agent selinexor in patients with heavily pre-treated, progressive gynecological cancers, oral selinexor showed promising anti-tumor activity or disease control across ovarian, endometrial and cervical cancers with disease control rates of up to 62% and several patients remaining on study for up to 12 months. In a Phase 1 clinical trial evaluating the activity of selinexor in Asian patients with advanced malignancies, single-agent selinexor demonstrated anti-tumor activity across a variety of malignancies in this patient population.

"We are excited by the activity observed to date with single agent oral selinexor in solid tumors, including the meaningful disease control rates observed in several different heavily pretreated gynecological cancer populations," said Sharon Shacham, PhD, MBA, President and Chief Scientific Officer of Karyopharm. "In addition, promising anti-tumor activity of selinexor was observed in Asian patients with highly refractory tumors including thymoma, *KRAS* mutant colorectal cancer and diffuse large B-cell lymphoma, consistent with our data in non-Asian patients. These data extend the potential utility of selinexor to the treatment of additional diseases and patient populations."

In a poster presented on Saturday, May 30, 2015, entitled, "Preliminary phase II results of selinexor, an oral selective inhibitor of nuclear export in patients with heavily pretreated gynecological cancers," Dr. Ignace Vergote (University Hospital Leuven) and colleagues from additional centers described data from an ongoing Phase 2 study of single-agent selinexor, including durable anti-cancer activity and disease control of up to 6 months, in patients with advanced ovarian, endometrial and cervical cancers no longer responding to prior therapies. All data are as of May 10, 2015.

Cancer Type	N	DCR	PR	SD &#62 12 weeks	PD
Ovarian	33	18 (55%)	4 (12%)	14 (42%)	15 (45%)
Endometrial	12	8 (67%)	2 (17%)	6 (50%)	4 (33%)
Cervical	18	7 (39%)	1 (6%)	6 (33%)	11 (61%)

Responses adjusted according to the Response Evaluation Criteria in Solid Tumors (RECIST v1.1).

DCR=Disease Control Rate (PR+SD), PR=Partial Response, SD=Stable Disease, PD=Progressive Disease

- Median PFS were approximately 177 days for endometrial cancers, 84 days for ovarian cancer, and 66 days for cervical cancer. Several patients remain on study for more than 6-11 months without clinically significant cumulative toxicities.
- Most common adverse events, including nausea, anorexia, fatigue and thrombocytopenia, were typically Grades 1 or 2 and attenuated over time and/or responded to supportive care.
- Circulating Tumor Cells (CTCs) were evaluated as a predictive marker for tumor response. CTC were collected at baseline and again following selinexor treatment. Patients with no CTCs at baseline responded better to selinexor treatment with only 8% progressive disease and a median of 118 days on study as compared to patients with CTC, of whom 70% had progressive disease and a median of 47 days on study.

In a poster presented on Saturday, May 30, 2015, entitled, "Phase I study of the safety and tolerability of the Exportin 1 (XPO1) inhibitor Selinexor (SXR) in Asian patients (pts) with advanced solid cancers," Dr. David Tan (National University Cancer Institute in Singapore) and colleagues described the activity and tolerability profile of single-agent selinexor in Asian patients with promising anti-tumor activity observed in Asian patients with heavily pretreated tumors including thymoma, *KRAS* mutant colorectal cancer and DLBCL.

- 19 patients receiving escalating doses of selinexor across three schedules were evaluable. Two of three patients with refractory diffuse large B-cell lymphoma (DLBCL) achieved a partial response and eight of 16 patients representing a variety of solid tumors including colorectal, pancreas, squamous cell tongue, non-small cell lung, ovarian and hepatocellular carcinoma achieved stable disease.

About Selinexor

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, 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 900 patients have been treated with selinexor in company- and investigator-sponsored Phase 1 and Phase 2 clinical trials in advanced hematologic malignancies and solid tumors. Karyopharm has initiated three registration-directed clinical trials of selinexor, including one in older patients with acute myeloid leukemia (SOPRA), one in patients with Richter's transformation (SIRRT) and one in patients with diffuse large B-cell lymphoma (SADAL). A single-arm trial of selinexor in patients with multiple myeloma (STORM) that is also intended to be registration-directed was initiated in May 2015. In solid tumors, Karyopharm plans to initiate a registration-directed trial of selinexor to treat liposarcoma during the second half of 2015. Additional Phase 1 and Phase 2 studies are ongoing or currently planned, including multiple studies in combination with one or more approved therapies in a variety of tumor types to further inform the company's clinical development priorities for selinexor. 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 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). 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 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 Quarterly Report on Form 10-Q for the quarter ended March 31, 2015, which is on file with the Securities and Exchange Commission (SEC) as of May 11, 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.

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