Karyopharm Announces Additional Orphan Designations Granted for Selinexor (KPT-330) by European Commission

NEWTON, Mass., Dec. 1, 2014 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a clinical-stage pharmaceutical company, today announced that its lead drug candidate, Selinexor (KPT-330), has received orphan drug designation from the European Medicines Agency (EMA) of the European Commission for the treatment of chronic lymphocytic leukemia (CLL) and small lymphocytic lymphoma (SLL), including Richter's Transformation, and for the treatment of multiple myeloma.

Selinexor has previously received orphan designation in acute myeloid leukemia (AML) and diffuse large B-cell lymphoma (DLBLC) from both the EMA and the U.S. Food and Drug Administration (FDA). Orphan designation was created to encourage the development of drugs which may provide significant benefit to patients suffering from rare diseases.

"The granting of orphan designation by the EMA for these hematologic malignancies represents a further significant achievement for our Selinexor development program," commented Dr. Sharon Shacham, President and Chief Scientific Officer of Karyopharm. "There continues to be limited treatment options for patients with these serious blood cancers, particularly for those patients who fail to achieve and maintain responses on available therapies. We are excited about the prospects for oral Selinexor's novel mechanism of action to potentially treat these challenging conditions, either alone or in combination with other therapies."

Orphan designation by the EMA is granted to promote the development of drugs that target rare life-threatening or debilitating conditions and that are expected to provide significant therapeutic advantage over existing treatments. Orphan designation qualifies a company for benefits that include targeted scientific advice from the EMA regarding drug development and ten years of market exclusivity following marketing approval.

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 500 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. Additional registration-directed clinical trials in hematological indications, including one in patients with diffuse large B-cell lymphoma (DLBCL) which is expected to begin in Q4 2014, are planned. At least one additional registration-directed clinical trial in a hematologic or solid tumor indication is also planned for the first half of 2015. 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 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 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, the European Medicines Agency 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.

CONTACT: Justin Renz

(617) 658-0574

jrenz@karyopharm.com

Gina Nugent

(617) 460-3579

nugentcomm@aol.com
