

# Karyopharm Initiates Registration-Directed Clinical Study of Selinexor (KPT-330) in Patients With Richter's Transformation

NEWTON, Mass., Nov. 10, 2014 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a clinical-stage pharmaceutical company, today announced the initiation of its Phase 2 study of Selinexor (KPT-330) in patients with Richter's transformation (SIRRT) that is relapsed after or refractory to chemotherapy. This multi-center study of Selinexor, one of the company's novel, oral Selective Inhibitor of Nuclear Export / SINE™ compounds, will enroll approximately 50 patients in approximately 35 sites worldwide.

This single arm, open-label, Phase 2 study will evaluate the safety and efficacy of Selinexor given orally at 60 mg/m<sup>2</sup> twice weekly. Overall response rate is the primary endpoint of this study, which was designed based on data from the ongoing Phase 1 study of Selinexor in patients with advanced hematologic malignancies, including Richter's. This study is expected to take 2 years to complete.

"Richter's Transformation is a rare condition in which chronic lymphocytic leukemia transforms into a fast-growing type of aggressive lymphoma. Treatment options for these patients are limited and prognosis is generally poor, with median survival less than 10 months from diagnosis," said Sharon Shacham, PhD, MBA, President and Chief Scientific Officer of Karyopharm. "Anti-cancer activity observed to in patients with Richter's Transformation enrolled in our Phase 1 Selinexor study in advanced hematologic malignancies gives us encouragement that this registration-direction will support Selinexor as a treatment option for this group of patients."

"The initiation of our second registration-directed clinical study with Selinexor represents additional progress toward our goal of accelerating drug development for patients with severe hematologic indications with high unmet medical need," said Michael G. Kauffman, MD, PhD, Chief Executive Officer of Karyopharm. "In addition, we are continuing development efforts with Selinexor, both as a single agent and in combination with current standard-of-care therapy, in a number of other hematologic and solid tumor indications."

## About Richter's Transformation

Richter's Transformation describes the appearance of an aggressive non-Hodgkin's lymphoma (NHL) in patients with chronic lymphocytic leukemia (CLL), or less commonly in patients with indolent lymphomas. There are about 1500 new cases of Richter's Transformation in the United States each year, with a similar number in the European Union. Approximately 10% of patients with CLL will develop Richter's Transformation over the course of their disease, and the transformation is usually associated with longer treatment duration and certain genetic abnormalities. Although Richter's Transformation most closely resembles diffuse large B-cell lymphoma (DLBCL), it is genetically distinct and typically resistant to therapies that are highly active against DLBCL. There are currently no drugs approved by the U.S. Food and Drug Administration, European Medicines Agency or Health Canada for the treatment of Richter's Transformation.

## 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](http://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](http://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 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.

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