

# Karyopharm Initiates Registration-Directed, Randomized Study of Selinexor (KPT-330) in Older Patients With Relapsed/Refractory Acute Myeloid Leukemia (AML)

NATICK, Mass., June 24, 2014 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), 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, today announced the initiation of its Phase 2 study of Selinexor (KPT-330) in patients 60 years of age or older with relapsed or refractory acute myeloid leukemia (AML) who are ineligible for intensive chemotherapy and/or transplantation. This Selinexor in Older Patients with Relapsed/Refractory AML (SOPRA) study is a randomized trial of Selinexor, the company's novel oral Selective Inhibitor of Nuclear Export (SINE) compound, versus physician's choice, and will be conducted at approximately 40 sites worldwide including sites in the United States, Canada, Europe and Israel.

In SOPRA, 150 patients with AML which has relapsed after, or was refractory to, first line therapy will be randomized in a 2:1 fashion to Selinexor provided orally twice per week at a dose of 55mg/m<sup>2</sup> versus one of four physician choices. Physician choices include best supportive care (BSC) alone, or BSC plus either azacytidine (Vidaza), decitabine (Dacogen), or low dose cytosine arabinoside (LD-AraC). Overall survival is the primary endpoint. SOPRA was designed based on data from the ongoing Phase 1 study of Selinexor in patients with advanced hematologic malignancies, including AML. SOPRA is expected to take approximately two years to complete.

William Blum, MD, Associate Professor in the Division of Hematology at The Ohio State University and a member of the Molecular Biology and Cancer Genetics Program at Ohio State's Comprehensive Cancer Center - James Cancer Hospital and Solove Research Institute, the lead investigator for the study, commented, "Older patients with relapsed AML have very limited treatment options. Because Selinexor is taken orally, and based on responses previously seen in older patients with heavily pretreated AML, we look forward to the results of this study."

Dr. Sharon Shacham, Founder, President and CSO of Karyopharm, said, "The initiation of this first registration-directed study of Selinexor represents a major milestone for Karyopharm. Based on data from our ongoing studies, we have focused on diseases with very high unmet medical need for our initial registration studies. We look forward to these results, and to potentially making Selinexor available to patients with this difficult to treat malignancy."

## About Selinexor

Selinexor (KPT-330) is a first-in-class, oral Selective Inhibitor of Nuclear Export (SINE) compound. Selinexor functions by binding with 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. To date, over 330 patients have been treated with Selinexor in Phase 1 and 2 trials in advanced hematologic malignancies and solid tumors. Additional Phase 1 and Phase 2 studies are ongoing or currently planned and three registration-directed clinical trials in hematological indications are expected to begin enrollment during 2014. 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 first-in-class drugs directed against nuclear transport targets for the treatment of cancer and other major diseases. SINE compounds have shown biological activity in models of cancer, autoimmune disease, certain viruses, and wound-healing. Karyopharm was founded by Dr. Sharon Shacham and is located in Natick, Massachusetts.

## 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, except as required by law, 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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