

# Karyopharm Announces Second Orphan Drug Designation for Selinexor in Diffuse Large B-Cell Lymphoma (DLBCL)

NATICK, Mass., May 20, 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 that its lead drug candidate, Selinexor (KPT-330), has received orphan drug designation from the U.S. Food and Drug Administration (FDA) for the treatment of Diffuse Large B-Cell Lymphoma (DLBCL). The designation is designed to encourage the development of drugs which may provide significant benefit to patients suffering from rare diseases.

"The granting of Orphan Drug Designation by the FDA for DLBCL is another significant milestone in the Selinexor development program," commented Dr. Sharon Shacham, Founder, CSO and President of Karyopharm. "There are limited treatment options for patients with relapsed or refractory DLBCL, with no new agents approved for this indication over the past two decades. Many patients relapse after responding to multi-agent first-line therapy. The fundamental treatment of DLBCL has changed little in the past two decades, with no new or targeted agents approved for this indication. Accordingly, we are excited about the prospects for Selinexor's novel mechanism of action to potentially treat this patient population, either alone or in combination with other therapies."

Orphan designation by the FDA is granted to promote the development of drugs that target conditions affecting 200,000 or fewer U.S. patients annually and that are expected to provide significant therapeutic advantage over existing treatments. Orphan designation qualifies a company for benefits that apply across all stages of drug development, including an accelerated approval process, seven years of market exclusivity following marketing approval, tax credits on U.S. clinical trials, eligibility for orphan drug grants, and a waiver of certain administrative fees.

Michael G. Kauffman, MD, PhD, Karyopharm's Chief Executive Officer said, "We are encouraged by the response data in patients with DLBCL who have received Selinexor in our ongoing Phase 1 clinical trial in advanced hematological malignancies. We look forward to the commencement of additional trials in patients with DLBCL, including a registration-directed clinical trial of Selinexor and investigator-sponsored combination studies of Selinexor. We plan to present updated clinical data for Selinexor across multiple indications, including DLBCL, at ASCO 2014."

Diffuse large B-cell lymphoma, or DLBCL, is a form of Non-Hodgkin lymphoma, or NHL, and is the most common of the aggressive NHLs, accounting for up to 30% of newly-diagnosed cases of NHL in the United States. According to the American Cancer Society, about 70,800 patients will be diagnosed with NHL in the United States in 2014 and an estimated 18,990 patients will succumb to the disease. Accordingly, approximately 21,000 patients will be diagnosed with DLBCL in the United States in 2014. In patients who are less than 65 years old, and who have good organ function, high dose chemotherapy with stem cell transplantation can lead to cures in approximately 50% of these patients. Older patients relapsing after initial chemotherapy, and those relapsing after stem cell transplantation, have a very poor prognosis, and the expected survival of such patients is less than one year.

## About Selinexor

Selinexor (KPT-330) is a first-in-class, oral Selective Inhibitor of Nuclear Export (SINE) compound. Selinexor functions by binding to 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 300 patients have been treated with Selinexor in Phase 1 and Phase 2 clinical 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. For more information about Karyopharm, 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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