Karyopharm Receives Orphan Drug Designation for Acute Myeloid Leukemia (AML)

NATICK, Mass., May 19, 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 that its lead drug candidate, Selinexor (KPT-330) oral, has received orphan drug designation from the U.S. Food and Drug Administration (FDA) for the treatment of Acute Myeloid Leukemia (AML). The designation is designed to encourage the development of drugs which may provide significant benefit to patients suffering from rare diseases.

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.

In addition to enrolling AML patients in its ongoing Phase 1 clinical trials of Selinexor, Karyopharm's development plans in AML include a number of additional studies for Selinexor.

- Randomized, Registration-Directed Clinical Study - Initiation is expected during the second quarter of 2014. This randomized study of Selinexor will enroll patients 60 years of age or older with relapsed or refractory AML who are ineligible for intensive chemotherapy and/or transplantation.

- First Combination Study. In this combination study, patients with relapsed or refractory AML or newly diagnosed AML patients 60 years of age or older ineligible for intensive chemotherapy will receive decitabine intravenously on days 1-10 and Selinexor orally twice weekly beginning on day 11 of each 31-day cycle. The study is being conducted at The Ohio State University Comprehensive Cancer Center in up to 42 patients with a primary goal to determine the maximum tolerated dose and the recommended Phase 2/3 dose of this combination. The secondary goal of the study is to determine the response rates and duration of leukemia control.

- First Study in Pediatric Patients. This study aims to determine the oral dosing, toxicity and preliminary clinical activity of Selinexor in pediatric leukemia patients. It will enroll up to 28 children with relapsed or refractory acute lymphoblastic leukemia (ALL) or AML. The study is being led by Dana-Farber/Boston Children's Cancer and Blood Disorders Center and is supported in part by a grant from the William Lawrence & Blanche Hughes Foundation.

Dr. Sharon Shacham, Founder, CSO and President of Karyopharm, said, "We are very pleased to have received Orphan Drug Designation for Selinexor in the treatment of AML, which will help advance the development of Selinexor, our lead clinical asset. Currently, AML treatment options, especially in patients with relapsed and/or refractory disease who are not eligible for intensive chemotherapy, are extremely limited. Selinexor has the potential to be a significant addition to the therapeutic options for this population. We are excited about Selinexor's novel mechanism of action, as well as its potential ability to synergize with other treatments."

"The granting of Orphan Drug Designation by the FDA in acute myeloid leukemia is a significant milestone in the Selinexor development program. This is an important disease area with high unmet medical need," said Michael Kauffman, M.D., Ph.D., Karyopharm's Chief Executive Officer. "We look forward to sharing updated clinical data on Selinexor at upcoming medical meetings, including ASCO."

Acute myeloid leukemia (AML) is the most common form of acute leukemia in adults, accounting for over 80% of all acute leukemias in individuals over 18 years of age. According to the American Cancer Society, an estimated 18,860 people will be diagnosed with AML in the United States in 2014 and 10,460 patients will die of the disease. Although survival rates have almost doubled for AML in the youngest age group, there has been little improvement in survival for adults in the older age groups, with overall five-year survival rates still less than five percent. A subsequent analysis based on the National Cancer Institute's Surveillance, Epidemiology, and End Result (SEER) dataset including 19,000 AML patients provided similar results; although overall survival improved consistently over the past three decades in patients ages 65 to 74 years, with improvements in 12-month survival from 20% (1977-1986), to 25% (1987-1996), to 30% (1997-2006), respectively, survival rates

did not improve in patients 75 years of age or older. In this study, the highest age groups (75-85 years and ≥ 85 years) had the lowest survival rates, with no apparent improvement compared to previous years.

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.

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.

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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