Karyopharm Announces Orphan Designation Granted by FDA for Selinexor (KPT-330) in Multiple Myeloma

NEWTON, Mass., Jan. 8, 2015 (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 U.S. Food and Drug Administration (FDA) for the treatment of multiple myeloma. Orphan designation was created to encourage the development of drugs which may provide significant benefit to patients suffering from rare diseases.

Selinexor has previously received orphan designation in multiple myeloma from the European Medicines Agency (EMA). Orphan designation has also been granted in acute myeloid leukemia (AML) and diffuse large B-cell lymphoma (DLBCL) from both the FDA and the EMA. In addition, the EMA has previously granted orphan designation of selinexor for the treatment of chronic lymphocytic leukemia (CLL) and small lymphocytic lymphoma (SLL), including Richter's Transformation.

"Orphan Drug Designation by the FDA for multiple myeloma is another significant milestone in the selinexor development program," commented Dr. Sharon Shacham, President and Chief Scientific Officer of Karyopharm. "We are encouraged by the response and durability data demonstrated to date in patients with multiple myeloma who have received selinexor in Phase 1 and Phase 1/2 studies and look forward to the commencement of additional trials with selinexor in patients with multiple myeloma in the near term, including a potential registration-directed trial we expect to commence during the first half of 2015."

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.

About Selinexor

Selinexor (KPT-330) is a first-in-class, oral Selective Inhibitor of Nuclear Export / SINE™ compound. Selinexor functions by binding with and 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 550 patients have been treated with selinexor in Phase 1 and Phase 2 clinical trials in advanced hematologic malignancies and solid tumors. Karyopharm has initiated three registration-directed clinical trials of selinexor, one in older patients with acute myeloid leukemia, one in patients with Richter's transformation and one in patients with diffuse large B-cell lymphoma (DLBCL). Karyopharm plans to initiate a single-arm trial in multiple myeloma in the first half of 2015 that is intended to be registration-directed. Other potential registration-directed trials in hematological and solid tumor indications are being evaluated. 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 first-in-class 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 cancer, 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 Current Report on Form 8-K filed with the Securities and Exchange Commission (SEC) on January 5, 2015, 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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