Karyopharm Presents Hematologic Cancer Data on Lead Drug Candidate Selinexor at International Conference on Malignant Lymphoma

NEWTON, Mass., June 22, 2015 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a clinical-stage pharmaceutical company, today announced the presentation of positive clinical and preclinical data for its lead product candidate, selinexor (KPT-330), a first-in-class, oral Selective Inhibitor of Nuclear Export / SINE™ compound, at the 13thInternational Conference on Malignant Lymphoma (ICML) 2015 held June 17-20, 2015 in Lugano, Switzerland. In an ongoing Phase 1 clinical trial which included 14 evaluable, relapsed, refractory DLBCL patients with triple, double or single hit MYC, BCL2 and/or BCL6 translocations, selinexor demonstrated clinically meaningful activity with a 43% overall response rate (partial response or better). Responses included two complete responses (CR) and four partial responses (PR), while two additional patients achieved stable disease (SD). In preclinical models, selinexor demonstrated potency in double hit (DH)-DLBCL cell lines in vitro and in an aggressive patient-derived xenograft (PDX) model of triple hit (TH) DLBCL, with 84% tumor growth inhibition. DLBCL with MYC, BCL2 and/or BCL6 translocations is an area of significant unmet medical need associated with poor prognosis and no standard-of-care treatment options.

"These results, along with DLBCL clinical data recently presented at the European Hematology Association (EHA) 2015 Annual Meeting, further demonstrate the potential of selinexor to serve significant unmet needs including high-risk DLBCL," said Sharon Shacham, PhD, MBA, President and Chief Scientific Officer of Karyopharm. "These data demonstrate selinexor's potent activity and encouraging disease control in double-hit and other high-risk DLBCL, an area of significant unmet medical need given the poor prognosis and limited treatment options associated with these disease subtypes."

Selinexor data in DH-DLBCL were described during an oral presentation by Dr. Ramiro Garzon of Ohio State University on Saturday, June 20, entitled "Selinexor Shows Marked Activity in Double-Hit Diffuse Large B Cell Lymphoma (DLBCL) in Pre-Clinical Models and in Patients with Heavily Pre-Treated Relapsed / Refractory Double-Hit DLBCL". These data from an ongoing Phase 1 clinical study of single-agent selinexor in patients with diffuse large B-cell lymphoma were as of June 1, 2015, including the following highlights:

- Among 14 evaluable, heavily pre-treated, patients with relapsed, refractory DLBCL with TH, DH or single-hit (SH) MYC, BCL2 and/or BCL6 translocations treated with selinexor, the overall response rate (ORR) was 43%, including two CRs and four PRs, while two patients achieved SD.
- Responses included three out of five patients with TH or DH DLBCL and three out of nine SH DLBCL, for a total of six out of 14 patients achieving objective responses.
- Toxicities were similar to the other patients in the study and no clinically significant organ dysfunction or cumulative toxicities were observed.
- In preclinical models, selinexor was potently cytotoxic and reduced Myc, Bcl2 and Bcl6 protein levels in DH-DLBCL cell lines in vitro and selinexor was highly active in a PDX model of TH DLBCL with 84% tumor growth inhibition.

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 900 patients have been treated with selinexor in company- and investigator-sponsored Phase 1 and Phase 2 clinical trials in advanced hematologic malignancies and solid tumors. Karyopharm has initiated four registration-directed clinical trials of selinexor, including one in older patients with acute myeloid leukemia (SOPRA), one in patients with Richter's transformation (SIRRT) and one in patients with diffuse large B-cell lymphoma (SADAL). A single-arm trial of selinexor in patients with multiple myeloma (STORM) that is also intended to be registration-directed was initiated in May 2015. In solid tumors, Karyopharm plans to initiate a registration-directed trial of selinexor to treat liposarcoma during the second half of 2015. Additional Phase 1 and Phase 2 studies are ongoing or currently planned, including multiple studies in combination with one or more approved therapies in a variety of tumor types to further inform the company's clinical development priorities for selinexor. 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). In addition to single-agent activity against a variety of different human cancers, 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 PAK4 inhibitor, 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 Quarterly Report on Form 10-Q for the quarter ended March 31, 2015, which is on file with the Securities and Exchange Commission (SEC) as of May 11, 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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