

Karyopharm Initiates Clinical Trial of Oral Selinexor in Advanced Liposarcoma

NEWTON, Mass., Jan. 14, 2016 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a clinical-stage pharmaceutical company, announced today the initiation of Selinexor in Advanced Liposarcoma ("SEAL"), a new Phase 2/3 clinical trial with oral selinexor, the Company's first-in-class, oral Selective Inhibitor of Nuclear Export (SINE™) compound that inhibits exportin 1 (XPO1).

SEAL is a multi-center, randomized, double-blind, placebo-controlled Phase 2/3 clinical trial evaluating single-agent oral selinexor in patients with advanced unresectable dedifferentiated liposarcoma. Patients will be randomized to receive either 60mg of selinexor or placebo given twice weekly per six week cycle until progression or intolerability. Fifty patients are expected to be enrolled in the Phase 2 portion of the study, with the potential to increase enrollment in the Phase 3 portion following an interim analysis. The primary endpoint of progression free survival (PFS) was acceptable to the Food and Drug Administration (FDA). Top-line data from the Phase 2 portion of this study are expected in early 2017.

"The rationale for SEAL is based on data from a Phase 1b study demonstrating durable stable disease with single-agent selinexor in patients with liposarcoma and other sarcomas," said Mrinal M. Gounder, MD, Attending Physician, Sarcoma Service and Developmental Therapeutics Service, Memorial Sloan Kettering Cancer Center, and Lead Investigator of the SEAL trial.

"These data, presented at the American Society of Clinical Oncology (ASCO) 2015 Annual Meeting in June, showed durable responses in liposarcoma, leiomyosarcoma and other sarcomas. Patients with liposarcoma appeared to benefit the most with selinexor, showing an improvement in progression free survival compared to previous chemotherapies." In the Phase 1b study, selinexor showed longer disease control duration compared to the patient's most recent prior therapy, with 14 of 18 liposarcoma patients (78%) achieving stable disease, including six (43%) of these fourteen patients achieving stable disease for greater than four months.

"With a less-than-5% five-year survival rate for recurrent and high-grade forms of liposarcoma, there are few effective treatment options for these uncommon, difficult to treat tumors that arise from the body's fat tissue," said Sant P. Chawla MD, Director, Santa Monica Oncology Center, Sarcoma Oncology Center. "Extending progression free survival is an important goal for these patients, in whom the rapid progression of disease frequently translates into early mortality. I look forward to seeing how encouraging early results from selinexor in sarcomas, in which extended disease stabilization was observed, translate to this larger outcome study."

"With our pivotal clinical strategy for selinexor in hematologic malignancies moving toward several key top line readouts, initiation of the SEAL study signals an important expansion of this strategy into solid tumors," said Sharon Shacham, PhD, MBA, President and Chief Scientific Officer of Karyopharm. "We believe selinexor offers the potential to provide a still deeply underserved liposarcoma patient population with a new therapeutic option. We look forward to the advancement of this study and to the launch of additional randomized studies of selinexor in solid tumors, as well as hematologic malignancies, where high unmet medical needs persist."

About Liposarcoma

Liposarcoma arises from fat cells or their precursors and, according to the nonprofit organization SARC, the Sarcoma Alliance for Research through Collaboration, represents 18% of all soft tissue sarcoma, or an estimated 2,500 new cases per year in the United States. Liposarcoma most commonly occurs in the thigh, behind the knee, the groin, the gluteal area or behind the abdominal cavity (retroperitoneum). Soft tissue sarcomas can invade surrounding tissue and can metastasize (spread) to other organs of the body. Dedifferentiated liposarcoma is an aggressive form of soft tissue sarcoma that is resistant to both standard chemotherapy and radiation. Liposarcoma has a particularly high rate of recurrence following surgery, especially in cases involving the abdomen. Except for cases that cured with surgery, most patients with liposarcoma will succumb to this disease, and novel therapies are needed.

About Selinexor

Selinexor (KPT-330) is a first-in-class, oral Selective Inhibitor of Nuclear Export (SINE™) compound. Selinexor functions by binding to and inhibiting the nuclear export protein XPO1 (also called CRM1), leading to the accumulation of tumor suppressor proteins in the cell nucleus. This reinitiates and amplifies their tumor suppressor function and is believed to lead to the selective induction of apoptosis in cancer cells, while largely sparing normal cells. Over 1,400 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. In addition to the SEAL study, selinexor is being evaluated in several later-phase clinical trials, including one in older patients with acute myeloid leukemia (SOPRA), one in patients with Richter's transformation (SIRRT), one in patients with diffuse large B-cell lymphoma (SADAL) and a single-arm trial of selinexor and low-dose dexamethasone in patients with multiple myeloma (STORM). In early 2016, Karyopharm expects to initiate a Phase 2/3 clinical trial (SCORE) to evaluate the

combination of selinexor, carfilzomib and dexamethasone versus carfilzomib and dexamethasone in patients with relapsed/refractory multiple myeloma that were previously treated with a proteasome inhibitor and an immunomodulatory drug. 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 human cancers, SINE™ compounds have also shown biological activity in models of 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 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 September 30, 2015, which is on file with the Securities and Exchange Commission (SEC) as of November 9, 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.

Contact:

Justin Renz
(617) 658-0574
jrenz@karyopharm.com

Gina Nugent
(617) 460-3579
nugentcomm@aol.com

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