

Karyopharm Initiates STORM Clinical Trial of Oral Selinexor (KPT-330) in Multiple Myeloma

NEWTON, Mass., May 28, 2015 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a clinical-stage pharmaceutical company, today announced the initiation of the STORM study (Selinexor Treatment of Refractory Myeloma). STORM is a multi-center, single-arm Phase 2 study of selinexor (KPT-330) in heavily-pretreated patients with quad-refractory multiple myeloma. Selinexor, the company's lead, novel, oral Selective Inhibitor of Nuclear Export / SINE™ compound, will be evaluated in combination with low-dose dexamethasone in multiple myeloma patients whose disease was refractory to treatment with bortezomib (Velcade®), lenalidomide (Revlimid®), carfilzomib (Kyprolis®) and pomalidomide (Pomalyst®). The first cohort will enroll approximately 80 patients at about 30 sites, primarily in the United States, with ~25% of the patients also having received treatment with an anti-CD38 monoclonal antibody such as daratumumab. Depending on the results from this initial cohort, the trial may be expanded to include additional patients. Data from an expanded trial may support an accelerated approval of selinexor in refractory multiple myeloma. Selinexor received orphan drug designation from the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA) for multiple myeloma.

"We are very encouraged by the responses and durability demonstrated to-date with selinexor in combination with low-dose dexamethasone in patients with relapsed and refractory multiple myeloma and look forward to continuing to evaluate selinexor in this patient population," said Sharon Shacham, PhD, MBA, President and Chief Scientific Officer of Karyopharm. "Despite the approvals of a variety of new agents, myeloma remains a fatal disease with nearly all patients relapsing after all available therapies. Selinexor has demonstrated promising durability and early signs of potential synergy with some of the approved anti-myeloma agents. We look forward to presenting the data from STORM in the near future."

This open-label, single-arm Phase 2 study of selinexor in combination with low-dose dexamethasone will evaluate the safety and efficacy of a fixed dose of selinexor (80 mg) plus low dose dexamethasone (20 mg). Each compound will be given orally twice weekly to approximately 80 patients with multiple myeloma with quad-refractory multiple myeloma. In addition, patients will have received alkylating agents and glucocorticoids, and their myeloma must be refractory to their most recent therapy. Overall response rate (ORR) is the primary endpoint of the study and this endpoint has served as the basis for accelerated approvals in multiple myeloma for other agents. STORM was designed based on data from Karyopharm's Phase 1 study of selinexor in combination with low dose dexamethasone in relapsed/refractory multiple myeloma.

As of December 1, 2014, Phase 1/2 data from ten heavily pretreated myeloma patients (median of seven prior therapies), nine of whom were evaluable for response, demonstrated a 67% ORR (partial response or better) and an 89% clinical benefit rate (minimal response or better). The overall median duration of response, measuring time from response to progression, is approximately seven months.

"We are very excited and honored to partner with Karyopharm to help bring life-saving treatments to multiple myeloma patients who are most in need," said Walter M. Capone, President and Chief Executive Officer of the Multiple Myeloma Research Foundation (MMRF). "The MMRF recognized the potential of selinexor in 2010, and has offered support throughout the compound's lifecycle by first awarding Karyopharm a Biotech Investment Award (BIA) to help advance selinexor from preclinical analysis through the initiation of clinical testing in myeloma patients. Promising results spurred the Multiple Myeloma Research Consortium (MMRC) to launch a Phase 1 clinical trial in 2014 to assess selinexor in combination with Kyprolis® in patients relapsed/refractory myeloma. We are committed to continuing our partnership with Karyopharm on the STORM trial, and to rapidly advancing lifesaving treatments to patients, particularly those with few, if any, active therapy options."

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. With the initiation of the STORM study, Karyopharm has now commenced 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). In solid tumors, Karyopharm plans to initiate a registration-directed randomized trial of single agent 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 Multiple Myeloma

Multiple myeloma is the second most common hematological malignancy (after non-Hodgkin's lymphoma), representing 1% of all cancers and 2% of all cancer deaths. Despite the increased effectiveness of a variety of agents, including the availability of several newly approved therapies including immunomodulatory agents, proteasome inhibitors and histone deacetylase (HDAC) inhibitors, nearly all patients will eventually relapse with their disease becoming drug-resistant. The American Cancer Society estimates that 26,850 new cases of multiple myeloma will be diagnosed in the U.S. in 2015. With an estimated 11,240 deaths from multiple myeloma expected in the U.S. this year, there remains a significant unmet need for therapies to treat patients with relapsed and/or refractory multiple myeloma that has progressed on available agents.

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.

About The Multiple Myeloma Research Foundation

The Multiple Myeloma Research Foundation (MMRF) was established in 1998 as a 501(c)(3) non-profit organization by twin sisters Karen Andrews and Kathy Giusti, soon after Kathy's diagnosis with multiple myeloma. The mission of the MMRF is to relentlessly pursue innovative means that accelerate the development of next-generation multiple myeloma treatments to extend the lives of patients and lead to a cure. As the world's number-one private funder of multiple myeloma research, the MMRF has raised \$275 million since its inception and directs nearly 90% of total budget to research and related programming. For more information about the MMRF, visit www.themmr.org.

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 Annual 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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