

Karyopharm Outlines Key Selinexor Clinical Development Achievements

- Enrollment Complete in Phase 2b STORM Clinical Trial in Multiple Myeloma - - Patient Dosing Underway in Phase 1b Clinical Trial of Selinexor in Combination with Chemotherapeutic, Targeted and Immunotherapeutic Agents -

NEWTON, Mass., June 16, 2016 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a clinical-stage pharmaceutical company, today provided an update relating to certain clinical achievements for selinexor (KPT-330), the Company's lead, novel, oral Selective Inhibitor of Nuclear Export / SINE™ compound.

The Company announced that enrollment has been completed in the ongoing STORM (Selinexor Treatment of Refractory Myeloma) study, a Phase 2b, single-arm clinical trial evaluating the activity of selinexor in combination with low-dose dexamethasone for the treatment of refractory multiple myeloma (MM) ([NCT02336815](#)). In this study, selinexor is being evaluated in heavily-pretreated patients, which the Company refers to as having at least quad-refractory MM. These are patients who have received bortezomib (Velcade®) and carfilzomib (Kyprolis®), each of which is a proteasome inhibitor (PI), and lenalidomide (Revlimid®) and pomalidomide (Pomalyst®), each of which is an immunomodulatory agent (IMiD), and whose disease is refractory to at least one IMiD, at least one PI, and is refractory to their most recent therapy. Prior treatment regimens must have also included an alkylating agent and a glucocorticoid. At least 25 percent of patients in this study must have MM that is also refractory to an anti-CD38 monoclonal antibody, such as daratumumab (Darzalex™), which the Company refers to as having penta-refractory MM.

"The completion of enrollment for STORM is an important milestone for selinexor and we remain on track to report top-line results during mid-2016," said Sharon Shacham, PhD, MBA, President and Chief Scientific Officer of Karyopharm. "These results will allow us to determine the best path forward, which may be expansion of the trial to include additional patients, which, if positive, could potentially serve as the basis for accelerated approval for patients with refractory multiple myeloma. Despite the availability of many newly approved therapeutic agents, myeloma remains a fatal disease with nearly all patients relapsing after all available treatments."

STORM enrolled 79 patients at 29 sites, mostly in the United States. The primary endpoint of the STORM study is overall response rate (ORR). The trial has several secondary endpoints, including ORR in patients whose disease is relapsed/refractory to an anti-CD38 monoclonal antibody, duration of response and clinical benefit rate. The first 51 patients enrolled in the study were treated with 80 mg selinexor plus 20 mg dexamethasone twice weekly for three out of every four weeks or six doses per cycle. The remaining 28 patients were treated with continuous dosing or eight doses per cycle. Of the 79 patients enrolled in the first cohort, 49 patients have quad-refractory disease and 30 patients have penta-refractory disease. Karyopharm expects to report top-line results from these patients in mid-2016. Depending on the results, the trial may be expanded in additional patients with penta-refractory MM, where an unmet medical need remains, to further evaluate continuous dosing for potential regulatory submission as a basis for accelerated approval based on ORR.

Today, Karyopharm also announced dosing of the first patient in a new arm of an ongoing investigator-sponsored Phase 1b clinical trial evaluating selinexor in combination with chemotherapeutic, targeted and immune-oncology agents for the treatment of advanced solid tumor malignancies ([NCT02419495](#)). This open label study, which is being conducted at MD Anderson Cancer Center, is designed to evaluate the safety and tolerability of selinexor in combination with up to 13 separate chemotherapeutic, targeted and immune-oncology agents. The first patient was dosed in the new study arm which is evaluating selinexor in combination with the anti-PD-1 immunotherapy pembrolizumab (Keytruda®). Across all 13 arms, up to 470 patients are expected to be enrolled in the study with the primary endpoint being determination of the maximum tolerated dose (MTD) and secondary efficacy endpoints as measured by RECIST. Karyopharm expects to report top-line data from this study over the next 18 months.

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. 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 1,500 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. Selinexor

is being evaluated in several later-phase clinical trials, including one in older patients with acute myeloid leukemia (SOPRA), one in patients with diffuse large B-cell lymphoma (SADAL), one in patients with liposarcoma (SEAL) and a single-arm trial of selinexor and low-dose dexamethasone in patients with multiple myeloma (STORM). 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 and related 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 and combination activity against a variety of human cancers, SINE™ compounds have also shown biological activity in models of neurodegeneration, 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 March 31, 2016, which was filed with the Securities and Exchange Commission (SEC) on May 9, 2016, 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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