Multiple Myeloma Research Foundation (MMRF) and Karyopharm Collaboration Drives the Advancement of Selinexor, a First-In-Class, Next-Generation Myeloma Therapy

NORWALK, Conn. and NEWTON, Mass., Jan. 31, 2018 (GLOBE NEWSWIRE) -- The Multiple Myeloma Research Foundation (MMRF), the world's number one private funder of multiple myeloma research, and Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a clinical-stage pharmaceutical company, today highlighted the significant advancement of an innovative potential new treatment option, selinexor, Karyopharm's lead, novel, oral Selective Inhibitor of Nuclear Export (SINE) compound, while part of the MMRF's Biotech Investment Award (BIA) program. In 2011, under a research agreement with MMRF, Karyopharm was awarded $1 million in research funding and strategic support for the study of oral selinexor as a potential new treatment for patients with multiple myeloma (MM).

Since receiving the funding from the MMRF, oral selinexor has progressed from a promising preclinical compound and is now being evaluated in the pivotal Phase 3 BOSTON (Bortezomib, Selinexor and dexamethasone) study in combination with Velcade® (bortezomib) and low-dose dexamethasone in patients with relapsed or refractory MM who have had one to three prior lines of therapy, an area of significant unmet need. The positive clinical data reported in selinexor's lead indication of MM, combined with the robust ongoing global clinical development program, resulted in an exclusive license with Ono Pharmaceutical Co. Ltd., an established leader in the Japanese oncology market, for the rights to selinexor in a transaction valued at up to $193 million (USD). The agreement gives Ono the right to develop and commercialize selinexor for the diagnosis, treatment and/or prevention of all human oncology indications in Japan and certain other Asian countries. Under the research agreement with MMRF, Karyopharm is proud to make certain payments to MMRF up to a defined cap on sales or out-license revenue, with the first payment triggered by the execution of the partnership with Ono.

"Despite tremendous progress over the past several years, there remains a significant need for novel approaches and effective treatments for patients with multiple myeloma, particularly for those patients who have run out of available treatment options," said Paul A. Giusti, President and Chief Executive Officer of the MMRF. "At the MMRF, we are committed to ensuring that organizations have the funding and resources necessary to accelerate the availability of promising new therapies and are proud to have partnered with Karyopharm over the last several years to support the development of selinexor."

Sharon Shacham, PhD, MBA, President and Chief Scientific Officer of Karyopharm, commented, "The MMRF has been an invaluable strategic partner for several years and it is highly gratifying that their early support for oral selinexor would lead to a robust clinical development program in myeloma. We are pleased that the recent licensing transaction with a widely respected oncology leader in Japan, an important global territory, will provide a return on MMRF's investment in Karyopharm and allow further investments to treat myeloma. We look forward to continuing our long and constructive relationship with the MMRF for many years to come."

Karyopharm's other ongoing clinical trials in myeloma include the Phase 2b STORM (Selinexor Treatment of Refractory Myeloma) study evaluating oral selinexor in combination with low-dose dexamethasone in patients with heavily-pretreated MM, and the Phase 1b/2 STOMP (Selinexor and Backbone Treatments of Multiple Myeloma Patients) study evaluating oral selinexor and low-dose dexamethasone in combination with backbone therapies Velcade® (bortezomib), Pomalyst® (pomalidomide), Revlimid® (lenalidomide) or Darzalex® (daratumumab) in patients with heavily pretreated relapsed or

refractory MM.

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. To date, over 2,200 patients have been treated with selinexor, and it is currently being evaluated in several mid- and later-phase clinical trials across multiple cancer indications, including in multiple myeloma in a pivotal, randomized Phase 3 study in combination with Velcade® (bortezomib) and low-dose dexamethasone (BOSTON), in combination with low-dose dexamethasone (STORM) and backbone therapies (STOMP), and in diffuse large B-cell lymphoma (SADAL), and liposarcoma (SEAL), among others. Additional Phase 1, Phase 2 and Phase 3 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 Karyopharm's clinical development priorities for selinexor. Additional clinical trial information for selinexor is available at www.clinicaltrials.gov.

About the Multiple Myeloma Research Foundation

The mission of the Multiple Myeloma Research Foundation (MMRF) is to find a cure for multiple myeloma by relentlessly pursuing innovation that accelerates the development of next-generation treatments to extend the lives of patients. Founded in 1998 by Kathy Giusti, a multiple myeloma patient, and her twin sister Karen Andrews as a 501(c) (3) nonprofit organization, the MMRF is a world-recognized leader in cancer research. Together with its partners, the MMRF has created the only end-to-end solution in precision medicine and the single largest genomic dataset in all cancers. The MMRF continues to disrupt the industry today, as a pioneer and leader at the helm of new research efforts. Since its inception, the organization has raised over $400 million and directs nearly 90% of the total funds to research and related programs. To learn more, visit www.themmrf.org.

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, which was founded by Dr. Sharon Shacham, currently has several investigational programs in clinical or preclinical development. 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 payments to MMRF, the potential to receive payments under the license agreement with Ono; the success of Karyopharm’s arrangement with Ono and the parties’ ability to work effectively together; the therapeutic potential of and potential clinical development plans for Karyopharm's drug candidates, including enrollment of certain trials and the timing of 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 Karyopharm's current expectations. For example, there can be no guarantee that any of Karyopharm's SINE compounds, including selinexor (KPT-330), 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: the ability of Karyopharm or Ono to fully perform their respective obligations under the license agreement; 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 impact volatility in currency exchange rates; 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, including with respect to the need for additional clinical studies; 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, 2017, which was filed with the Securities and Exchange Commission (SEC) on November 2, 2017, 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, except as required by law, 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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