

Karyopharm Initiates Rolling Submission of New Drug Application to U.S. Food and Drug Administration for Selinexor as a Treatment for Patients with Penta-Refractory Multiple Myeloma

-- Company Plans to Complete Submission During the Second Half of 2018 --

NEWTON, Mass., July 18, 2018 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a clinical-stage pharmaceutical company, today announced that the Company has initiated a rolling submission of a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) seeking accelerated approval for selinexor, its novel, oral SINE compound, as a new treatment for patients with penta-refractory multiple myeloma. Patients with penta-refractory myeloma have previously received the two proteasome inhibitors (PIs), Velcade® (bortezomib) and Kyprolis® (carfilzomib), the two immunomodulatory drugs (IMiDs), Revlimid® (lenalidomide) and Pomalyst® (pomalidomide), and the anti-CD38 monoclonal antibody Darzalex® (daratumumab), and their disease is refractory to at least one PI, at least one IMiD, Darzalex and their most recent therapy. The Company expects to complete the NDA submission during the second half of 2018. Selinexor has received both Orphan Drug and Fast Track designations from the FDA for this indication.

“We believe that selinexor has the potential to address the critical unmet need for patients with highly resistant, penta-refractory myeloma, where the disease is no longer responsive to standard approved therapies,” said Sharon Shacham, PhD, MBA, Founder, President and Chief Scientific Officer of Karyopharm. “The commencement of this rolling submission for the oral selinexor NDA marks the first-ever application for regulatory approval of an Exportin 1 (XPO1) inhibitor and represents a major milestone for the Company. We are proud of the positive Phase 2b STORM study results underlying this application and we will work expeditiously to complete the submission this year.”

Pending marketing approval by the FDA, Karyopharm plans to commercialize selinexor in the U.S. The Company also plans to submit a Marketing Authorization Application to the European Medicines Agency in early 2019 with a request for conditional approval.

About Selinexor

Selinexor 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 2,500 patients have been treated with selinexor. In April 2018, Karyopharm reported positive top-line data from the Phase 2b STORM study evaluating selinexor in combination with low-dose dexamethasone in patients with penta-refractory multiple myeloma. Selinexor has been granted Orphan Drug Designation in multiple myeloma and Fast Track designation for the patient population evaluated in the STORM study. Karyopharm has initiated a rolling submission for a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA), with a request for accelerated approval for oral selinexor as a new treatment for patients with penta-refractory multiple myeloma and expects the submission to be complete during the second half of 2018. The Company also plans to submit a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) in early 2019 with a request for conditional approval. Selinexor is also being evaluated in several other 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), as a potential backbone therapy in combination with approved therapies (STOMP), in diffuse large B-cell lymphoma (SADAL), liposarcoma (SEAL), and an investigator-sponsored study in endometrial cancer (SIENDO), among others. Additional Phase 1, Phase 2 and Phase 3 studies are ongoing or currently planned, including multiple studies in combination with 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.

Further Information About Potential Accelerated Approval for Selinexor in Multiple Myeloma

The FDA instituted its Accelerated Approval Program to allow for expedited approval of drugs that treat serious conditions and that fill an unmet medical need based on a surrogate endpoint or an intermediate clinical endpoint thought to predict clinical benefit, like overall response rate (ORR). Accelerated approval is available only for drugs that provide a meaningful therapeutic benefit over existing treatments at the time of consideration of the application for accelerated approval, which the FDA has reiterated in its feedback to the Company. Particularly in disease areas with multiple available and potential new therapies, such as multiple myeloma, accelerated approval carries a high regulatory threshold. Consistent with its general guidance, the FDA has noted to the Company its preference for randomized studies geared toward full approval, which the Company has

undertaken with the ongoing pivotal, Phase 3 BOSTON study, and has reminded the Company that accelerated approval requires patients to have exhausted all available approved therapies. FDA's Fast Track designation is available to therapeutics treating an unmet medical need in a serious condition; the Company has received Fast Track designation from the FDA specifically for the population treated in the STORM trial. In light of this recognition that the STORM patient population represents an unmet medical need and the positive top-line data reported in April 2018, the Company believes that the STORM study should support its request to the FDA for accelerated approval.

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 the submissions to regulatory authorities, including the anticipated timing of such submissions, and the potential availability of accelerated approval pathways, the therapeutic potential of and potential clinical development plans for Karyopharm's drug candidates, especially selinexor, and the plans for commercialization. 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 drug candidates, including selinexor, will successfully complete necessary clinical development phases, that development of any of Karyopharm's drug candidates will continue or that any feedback from regulatory authorities will ultimately lead to the approval of selinexor or any of Karyopharm's other drug candidates. 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, 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 March 31, 2018, which was filed with the Securities and Exchange Commission (SEC) on May 10, 2018, 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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Source: Karyopharm Therapeutics Inc.

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