

# Karyopharm Announces Partial Clinical Hold to Pause Enrollment in Selinexor Trials

**All Currently Enrolled Patients with Stable Disease or Better Can Continue Receiving Selinexor  
Company Has Amended Investigator's Brochure and Informed Consent Documents as Requested by  
FDA  
Company Expects Timelines for Both Ongoing and Planned Studies to Remain Materially Unchanged**

NEWTON, Mass., March 10, 2017 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a clinical-stage pharmaceutical company, today announced that it has received written notice from the U.S. Food and Drug Administration (FDA) that its clinical trials for selinexor (KPT-330) have been placed on partial clinical hold. While the partial clinical hold remains in effect, patients with stable disease or better may remain on selinexor therapy. No new patients may be enrolled until the partial clinical hold is lifted.

The FDA has indicated that the partial clinical hold is due to incomplete information in the existing version of the investigator's brochure (IB), including an incomplete list of serious adverse events (SAEs) associated with selinexor. At the FDA's request, Karyopharm has amended the IB and updated the informed consent documents accordingly and has submitted such documents to the FDA as requested. The partial clinical hold is not the result of any patient death or any new information regarding the safety profile of selinexor. To date, more than 1,900 patients have been treated with selinexor in clinical trials across a variety of hematological and solid tumor malignancies.

As of Friday, March 10, 2017, Karyopharm had provided all requested materials to the FDA believed to be required to lift the partial clinical hold. By regulation, the FDA has 30 days from receipt of Karyopharm's submission to notify the company whether the partial clinical hold is lifted. Karyopharm is working diligently with the FDA to seek the release of the partial clinical hold and resume enrollment in its selinexor clinical trials as expeditiously as possible. Karyopharm believes that its previously disclosed enrollment rates and timelines for its ongoing trials will remain materially unchanged.

## 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 1,900 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 combination with low-dose dexamethasone (STORM) and backbone therapies (STOMP), and in diffuse large B-cell lymphoma (SADAL), and liposarcoma (SEAL), among others. Karyopharm plans to initiate a pivotal randomized Phase 3 study of selinexor in combination with bortezomib (Velcade®) and low-dose dexamethasone (BOSTON) in patients with multiple myeloma in early 2017. 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 the Company's clinical development priorities for selinexor. Additional clinical trial information for selinexor is available at [www.clinicaltrials.gov](http://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, 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](http://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 anticipated impact of the partial clinical hold, timing of FDA review of Karyopharm's response, Karyopharm's plans for obtaining the

release of the partial clinical hold, therapeutic potential of and potential clinical development plans for Karyopharm's drug candidates, including the timing of initiation of and enrollment in certain 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 the FDA will release the partial clinical hold in a timely manner or at all, 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: 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 FDA 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, 2016, which was filed with the Securities and Exchange Commission (SEC) on November 7, 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.

Velcade® is a registered trademark of Takeda Pharmaceutical Company Limited

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