



## Karyopharm Announces FDA Extension of Review Period for Selinexor New Drug Application

March 14, 2019

- PDUFA Action Date Extended by Three Months to July 6, 2019 -
- FDA Has Requested Additional Existing Information for Review -

NEWTON, Mass., March 14, 2019 /PRNewswire/ -- Karyopharm Therapeutics Inc. (Nasdaq: KPTI), a clinical-stage pharmaceutical company, today announced that the U.S. Food and Drug Administration (FDA) has extended the Prescription Drug User Fee Act (PDUFA) action date for the New Drug Application (NDA) for selinexor. The NDA, which is currently under Priority Review by the FDA, is seeking accelerated approval for selinexor in combination with dexamethasone for the treatment of patients with relapsed refractory multiple myeloma who have received at least three prior therapies and whose disease is refractory to at least one proteasome inhibitor (PI), one immunomodulatory agent (IMiD), and one anti-CD38 monoclonal antibody. The previously disclosed April 6, 2019 PDUFA date has been extended by three months to July 6, 2019.

On February 26, 2019, the FDA's Oncologic Drugs Advisory Committee (ODAC) met to discuss the selinexor NDA and voted 8 to 5 recommending that the FDA wait for the results from Karyopharm's randomized, open-label, Phase 3 BOSTON study evaluating selinexor in patients with relapsed or refractory multiple myeloma, before making a final decision regarding approval. Although the FDA considers the recommendation of this panel, the final decision regarding the approval of the product is made by the FDA solely, and the recommendations by the panel are non-binding.

Following the ODAC meeting, at the FDA's request, Karyopharm submitted additional, existing clinical information as an amendment to the NDA, which allowed the FDA to extend the PDUFA action date by three months. "We look forward to the continued collaboration with FDA in trying to meet the needs of patients with relapsed refractory multiple myeloma," said Sharon Shacham, PhD, MBA, Founder, President and Chief Scientific Officer of Karyopharm.

### 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. In 2018, Karyopharm reported positive data from the Phase 2b STORM study evaluating selinexor in combination with low-dose dexamethasone in patients with triple class refractory multiple myeloma who have been previously exposed to all five of the most commonly prescribed anti-myeloma therapies currently available. Selinexor has been granted Orphan Drug Designation in multiple myeloma and Fast Track designation for the patient population evaluated in the STORM study. Karyopharm's New Drug Application (NDA) has been accepted for filing and granted Priority Review by the FDA, and oral selinexor is currently under review by the FDA as a possible new treatment for patients with triple class refractory multiple myeloma. The Company has also submitted a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) with a request for conditional approval and was granted accelerated assessment. Selinexor is also being studied in patients with relapsed or refractory diffuse large B-cell lymphoma (DLBCL). In 2018, Karyopharm reported positive top-line results from the Phase 2b SADAL study evaluating selinexor in patients with relapsed or refractory DLBCL after at least two prior multi-agent therapies and who are ineligible for transplantation, including high dose chemotherapy with stem cell rescue. Selinexor has received Fast Track designation from the FDA for the patient population evaluated in the SADAL study. 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 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](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 our expectations relating to submissions to, and the review and potential approval of selinexor by, regulatory authorities, including the anticipated timing of such submissions and actions, 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, many of which are beyond Karyopharm's control, that may cause actual events or results to differ materially from Karyopharm's current expectations. For example, there can be no guarantee that regulators will agree that selinexor qualifies for accelerated approval in the U.S. or conditional approval in the E.U. as a result of our clinical data, including the data from the STORM study in patients with triple class refractory myeloma or the SADAL study in patients with relapsed or refractory

DLBCL, or that any of Karyopharm's drug candidates, including selinexor, will successfully complete necessary 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, 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 Annual Report on Form 10-K for the year ended December 31, 2018, which was filed with the Securities and Exchange Commission (SEC) on February 28, 2019, 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.

Velcade® is a registered trademark of Takeda Pharmaceutical Company Limited

 View original content: <http://www.prnewswire.com/news-releases/karyopharm-announces-fda-extension-of-review-period-for-selinexor-new-drug-application-300812840.html>

SOURCE Karyopharm Therapeutics Inc

Investors: Karyopharm Therapeutics Inc., Ian Karp, Vice President, Investor and Public Relations, 857-297-2241 | [ikarp@karyopharm.com](mailto:ikarp@karyopharm.com); Media: Argot Partners, David Rosen, 212-600-1902 | [david.rosen@argotpartners.com](mailto:david.rosen@argotpartners.com)