

Karyopharm to Present Selinexor Phase 2b SADAL Data at the American Association for Cancer Research Annual Meeting 2017

Abstract Published Today Highlighting Interim Phase 2b SADAL Results in Patients with Relapsed or Refractory DLBCL

Overall Response Rate Greater Than 28%, along with Additional Updated Data, to Be Presented in a Late-Breaking Poster at the Meeting

NEWTON, Mass., March 31, 2017 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a clinical-stage pharmaceutical company, today announced that interim clinical data from its Phase 2b SADAL study evaluating lead product candidate, selinexor (KPT-330), an oral Selective Inhibitor of Nuclear Export / SINE™ compound, in patients with relapsed or refractory diffuse large B-cell lymphoma (DLBCL) will be presented in a late-breaking poster at the American Association for Cancer Research (AACR) Annual Meeting 2017 taking place April 1-5, 2017 in Washington, DC.

As previously disclosed on March 16, 2017, in the Phase 2b SADAL study, selinexor has demonstrated a greater than 28% overall response rate (ORR), the primary endpoint for the trial, in the first 63 patients with relapsed or refractory DLBCL based on independent central radiological review. The ORR quoted in the abstract which was published today was based on investigator assessed response in the first 65 patients (scans for two of the patients were not available for central radiological review for the poster data cutoff date of March 1, 2017). Additional updated data, including duration of response and tolerability data, will be presented at AACR 2017.

As previously stated, the response rates were similar across both arms of the study, but greater durability and chronic tolerability were observed in the 60mg arm. Based on these data, and in consultation with the U.S. Food and Drug Administration (FDA), Karyopharm is amending the SADAL study to become a single-arm trial evaluating single-agent selinexor at 60mg given twice weekly and plans to make other protocol amendments, including to reduce the 14-week treatment-free period to eight weeks in patients who achieved at least a PR on their most recent therapy. Karyopharm expects to enroll up to an additional 90 patients to the new 60mg cohort and to announce topline data for the SADAL study in mid-2018. The FDA agreed that the change to a single-arm study was reasonable and that the proposed trial design and indication appeared appropriate for accelerated approval, though the availability of accelerated approval will depend on the trial results and available therapies at the time of regulatory action.

Details for the SADAL Late-Breaking Poster Presentation at AACR 2017:

Title: [A Phase 2b randomized study of selinexor in patients with relapsed/refractory diffuse large B-cell lymphoma \(DLBCL\) demonstrates durable responses in both GCB and non-GCB subtypes](#)

Presenter: Marie Maerevoet, Institute Jules Bordet

Poster Board #: 13

Session: Phase I-III Clinical Trials and Pediatric Clinical Trials

Location: Convention Center, Halls A-C, Poster Section 33

Date and Time: Tuesday, April 4, 2017 from 1:00 PM - 5:00 PM ET

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.

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 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), 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, 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, 2016, which was filed with the Securities and Exchange Commission (SEC) on March 16, 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 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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