

# Karyopharm Presents Updated Phase 2 SAIL Relapsed/Refractory AML Clinical Data at the American Society of Hematology 2016 Annual Meeting

## **- Data Demonstrate Robust Response Rates Enabling Transplantation or Donor Lymphocyte Infusions in Patients with Heavily Pretreated Acute Myeloid Leukemia -**

NEWTON, Mass., Dec. 04, 2016 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a clinical-stage pharmaceutical company, today announced updated results from the Phase 2 SAIL study, in which deep responses to selinexor (KPT-330) allowed patients with heavily pretreated acute myeloid leukemia (AML) to proceed onto stem cell transplantation or donor lymphocyte transfusion, at the American Society of Hematology (ASH) 2016 annual meeting held December 3-6, 2016 in San Diego. Selinexor is the Company's lead, novel, oral Selective Inhibitor of Nuclear Export (SINE™) compound, in development for the treatment of AML and a variety of additional malignancies.

"These updated SAIL data, along with key presentations by Drs. Amy Wang, Bhavana Bhatnagar, and Kendra Sweet demonstrate the feasibility and tolerability of selinexor in combination with chemotherapy and other commonly-used agents in patients with AML," said Sharon Shacham, PhD, MBA, President and Chief Scientific Officer of Karyopharm. "With response rates that are superior to historical data, the clinical results presented at ASH this year demonstrate that selinexor combination regimens could become effective treatment options and serve as a bridge to stem cell transplantation even for patients suffering with relapsed/refractory AML."

### Updated Phase 2 SAIL Clinical Data in Refractory AML

In an oral presentation titled, "Phase II Results of Ara-C and Idarubicin in Combination with the Selective Inhibitor of Nuclear Export Compound Selinexor in Patients with Relapsed or Refractory AML," Walter Fiedler, MD, University Medical Center Hamburg, described data demonstrating that selinexor in combination with Ara-C and idarubicin is safe with no unexpected toxicities observed to date and has the potential to achieve significant response rates, particularly in a heavily pretreated patient population.

Among the 42 patients evaluable for safety (median of 2 prior treatment regimens, all including intensive chemotherapy), as of October 2016, the overall response rate (ORR, 4 patients excluded from evaluation due to early death) was 55% and included 10 (26%) complete remissions (CR) and 10 (26%) achieving complete remission with incomplete blood count recovery (CRI). Based on these data, Karyopharm believes that selinexor in combination with Ara-C and idarubicin may be an effective treatment option and serve as a bridge to stem cell transplantation for patients with relapsed/refractory AML. The most frequent Grade  $\geq 3$  non-hematologic adverse events (AEs) of this intensive chemotherapy-containing regimen were diarrhea (50%) and nausea (12%). The most common Grade  $\geq 3$  hematologic AEs were neutropenia (100%) and thrombocytopenia (100%). Two deaths occurred which were deemed possibly treatment-related. There was one reported case of systemic inflammatory response syndrome (SIRS; 2%) and one reported case of hemophagocytosis syndrome (2%).

### Other Key AML Data Presented at ASH 2016

In addition to updated data from the SAIL study, additional key AML abstracts include:

Oral Presentation Title: Combination of Selinexor with High-Dose Cytarabine and Mitoxantrone for Remission Induction in AML Is Feasible and Tolerable

Presenter: Amy Wang, University of Chicago

Publication ID: 212

Date and Time: Saturday, December 3, 2016; 4:15 PM PT

Summary: In this study, the combination of selinexor with high-dose cytarabine (HiDAC) and mitoxantrone is feasible and tolerable and the recommended phase 2 dose was identified as selinexor 80mg per day plus HiDAC and mitoxantrone. This regimen demonstrated an ORR of 68% in all 19 patients and 91% in patients with newly diagnosed AML. Based on these results, the combination of selinexor plus HiDAC and mitoxantrone warrant further investigation.

Poster Title: A Phase 1 Clinical Trial of Selinexor in Combination with Decitabine in Patients with Newly Diagnosed and Relapsed or Refractory AML

Presenter: Bhavana Bhatnagar, Ohio State University

Publication ID: 1651

Date and Time: Saturday, December 3, 2016; 5:30-7:30 PM PT

Summary: In this study, the combination of selinexor and decitabine produced a CR/CRi/mCR (marrow CR) rate of 80% in older untreated patients and 26.3% in relapsed/refractory AML (RRAML) patients. The total CR/CRi/mCR rate was 37.5%. Importantly, six of the 19 patients with relapsed/refractory disease underwent allogeneic stem cell transplant (four with no evidence of AML at the time of transplant). Selinexor plus decitabine is an active regimen in poor-risk AML patients and alternative dosing schedules to improve long-term tolerability, compliance and efficacy should be explored.

Poster Title: A Phase I Study of Selinexor in Combination with Daunorubicin and Cytarabine in Patients with Newly Diagnosed Poor-Risk AML

Presenter: Kendra Sweet, Moffitt Cancer Center, Tampa FL

Publication ID: 4040

Date and Time: Monday, December 5, 2016; 6:00-8:00 PM PT

Summary: Data from this Phase 1 trial suggest that oral selinexor (80mg) twice weekly can be safely administered in combination with induction chemotherapy with cytarabine and daunorubicin in patients with poor-risk AML, including elderly patients. Response rates were encouraging, with many elderly patients proceeding to transplant, suggesting this regimen warrants further investigation in this challenging population.

#### 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 1,800 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 acute myeloid leukemia (SOPRA), 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. The latest 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 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 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.

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<https://investors.karyopharm.com/2016-12-04-Karyopharm-Presents-Updated-Phase-2-SAIL-Relapsed-Refractory-AML-Clinical-Data-at-the-American-Society-of-Hematology-2016-Annual-Meeting>