

# Karyopharm to Present Data on Oncology Pipeline at the 2016 American Association for Cancer Research Annual Meeting

## **Two Late-Breaking Posters Featuring the Company's Lead Drug Candidate, Selinexor (KPT-330), and Second Generation SINE Compound KPT-8602 Seventeen Additional Poster Presentations Highlighting Key Scientific Findings for Selinexor and the Company's Earlier Stage Oncology Programs**

NEWTON, Mass., March 16, 2016 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a clinical-stage pharmaceutical company, today announced that 19 abstracts describing the Company's product candidates in development for hematological and solid tumor malignancies have been selected for presentation at the 2016 Annual Meeting of the American Association for Cancer Research (AACR) taking place April 16-20 in New Orleans. The abstracts, which represent both company- and investigator-sponsored studies, describe data related to the Company's lead product candidate, selinexor (KPT-330), an oral Selective Inhibitor of Nuclear Export / SINE™ compound, as well as the Company's earlier stage oncology programs including KPT-8602, a second generation SINE compound, and KPT-9274, an oral dual inhibitor of PAK4 and NAMPT. Updated and detailed results from these studies will be presented at the conference.

"The broad body of preclinical research to be presented at this year's AACR annual meeting highlights key findings from our oncology pipeline and provides important support for our ongoing clinical development programs," said Sharon Shacham, PhD, MBA, President and Chief Scientific Officer of Karyopharm. "We are particularly excited about the selinexor data in combination with other oncology therapies, including immunotherapies. These data continue to build upon a growing body of scientific evidence supporting selinexor's potential as an important backbone therapy in combination with a variety of other anti-cancer agents across multiple hematological and solid tumor malignancies. Beyond selinexor, we will also be presenting new data for KPT-8602, which recently entered the clinic, and KPT-9274, which we expect to enter the clinic by the middle of this year."

### Late-Breaking Poster Presentations:

- Title: Combination therapy of immune checkpoint and nuclear exporter inhibitors in a renal cell carcinoma mouse model

Author: Trott, University of California, Davis  
Section/Board: 10/10  
Date/Time: Monday, April 18, 8:00 AM - 12:00 PM

- Title: KPT-8602 is a second-generation XPO1 inhibitor with improved in vivo tolerability that demonstrates potent acute lymphoblastic leukemia activity

Author: Daelemans, Rega Institute of Medical Research, KU Lueven-University of Lueven, Belgium  
Section/Board: 12/17  
Date/Time: Monday, April 18, 1:00 PM - 5:00 PM

Poster Presentations on selinexor, Karyopharm's first-in-class, oral Selective Inhibitor of Nuclear Export / SINE™ compound include:

- Poster Title: [Selinexor, a selective inhibitor of nuclear export \(SINE\), enhances the in vivo efficacy of checkpoint blockade with antibodies targeting CTLA4 or PD-1/PD-L1 in melanoma](#)

Author: Farren, Ohio State University  
Section/Board: 26/3  
Date/Time: Monday, April 18, 1:00 PM - 5:00 PM

- Poster Title: [Selinexor, a selective inhibitor of nuclear export \(SINE\) compound, shows synergistic anti-tumor activity when combined with PD-1 blockade in a mouse model of colon cancer](#)

Author: Elloul, Karyopharm  
Section/Board: 21/11

Date/Time: Monday, April 18, 1:00 PM - 5:00 PM

- Poster Title: [Selinexor, a selective inhibitor of nuclear export \(SINE\) compound shows enhanced anti-tumor activity when combined with either venetoclax or bendamustine in diffuse large B cell lymphoma \(DLBCL\) mouse models](#)

Author: Elloul, Karyopharm

Section/Board: 18/18

Date/Time: Monday, April 18, 8:00 AM - 12:00 PM

- Poster Title: [Synergistic antitumor effect of selinexor, a selective inhibitor of nuclear export \(SINE\) compound and trastuzumab in a mouse model of breast cancer](#)

Author: Elloul, Karyopharm

Section/Board: 14/17

Date/Time: Wednesday, April 20, 8:00 AM - 12:00 PM

- Poster Title: [Synergistic antitumor effect of selinexor, a selective inhibitor of nuclear export \(SINE\) compound and panobinostat in a mouse model of multiple myeloma](#)

Author: Elloul, Karyopharm

Section/Board: 17/1

Date/Time: Wednesday, April 20, 8:00 AM - 12:00 PM

- Poster Title: [Targeting nuclear export for triple-negative breast cancer therapy](#)

Author: Petrocca, Boston University

Section/Board: 3/14

Date/Time: Sunday, April 17, 1:00 PM - 5:00 PM

- Poster Title: [Selective inhibitor of nuclear export \(SINE\) compounds prevent migration and proliferation of triple negative breast cancer \(TNBC\) cells by restoring expression of ARDC3](#)

Author: Soung, Stony Brook University

Section/Board: 31/4

Date/Time: Sunday, April 17, 1:00 PM - 5:00 PM

- Poster Title: [Targeting nuclear transport pathways to overcome endocrine resistance and recurrence](#)

Author: Kulkoyluoglu, University of Illinois at Urbana -Champaign

Section/Board: 1/16

Date/Time: Monday, April 18, 1:00 PM - 5:00 PM

- Poster Title: [Targeting XPO1 overexpression with selinexor disrupts the survivin pathway in neuroblastoma](#)

Author: Castellanos, Albert Einstein College of Medicine

Section/Board: 32/28

Date/Time: Monday, April 18, 1:00 PM - 5:00 PM

- Poster Title: [Selinexor inhibits NF- \$\kappa\$ B activity by sequestering I \$\kappa\$ B- \$\alpha\$  in the Nucleus and Blocking I \$\kappa\$ B- \$\alpha\$  degradation](#)

Author: Kashyap, Karyopharm

Section/Board: 9/3

Date/Time: Tuesday, April 19, 8:00 AM - 12:00 PM

- Poster Title: [Cytoplasmic localization of PU.1 with mutated NPM1 causes myeloid differentiation arrest](#)

Author: Gu, Cleveland Clinic

Section/Board: 8/6

Date/Time: Tuesday, April 19, 8:00 AM - 12:00 PM

- Poster Title: [Cell cycle specific effects and associated DNA damage of selective inhibitors of nuclear export \(SINE\)](#)

Author: Burke, University of Colorado

Section/Board: 14/16

Date/Time: Wednesday, April 20, 8:00 AM - 12:00 PM

Poster Presentations on KPT-9274, Karyopharm's oral dual inhibitor of PAK4 and NAMPT include:

- Poster Title: [In vivo efficacy of the PAK4 allosteric modulator KPT-9274 against a triple-negative breast cancer model](#)  
Author: Rane, Rutgers University  
Section/Board: 4/2  
Date/Time: Monday, April 18, 1:00 PM - 5:00 PM
- Poster Title: [The role of p21-activated kinase 4 \(PAK4\) in cancer stemness and epithelial-to-mesenchymal transition](#)  
Author: Azmi, Wayne State University  
Section/Board: 4/11  
Date/Time: Monday, April 18, 1:00 PM - 5:00 PM
- Poster Title: [KPT-9274 inhibits cellular NAD and synergizes with NAD depleting enzymes to induce cancer cell death](#)  
Author: Argueta, Karyopharm  
Section/Board: 17/6  
Date/Time: Tuesday, April 19, 8:00 AM - 12:00 PM
- Poster Title: [The PAK4 allosteric modulator \(KPT-9274\) attenuates the growth of renal cell carcinoma](#)  
Author: Aboud, University of California, Davis  
Section/Board: 17/16  
Date/Time: Tuesday, April 19, 1:00 PM - 5:00 PM
- Poster Title: [Co-administration of nicotinic acid \(NA\) enhances the therapeutic index of KPT-9274 in cancer cells](#)  
Author: Senapedis, Karyopharm  
Section/Board: 20/22  
Date/Time: Wednesday, April 20, 8:00 AM - 12:00 PM

#### 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. Over 1,400 patients have been treated with selinexor in company and investigator-sponsored Phase 1 and Phase 2 clinical trials in advanced hematologic malignancies and solid tumors. Selinexor is being evaluated in several later-phase clinical trials, including one in older patients with acute myeloid leukemia (SOPRA), one in patients with Richter's transformation (SIRRT), one in patients with diffuse large B-cell lymphoma (SADAL), one in patients with liposarcoma (SEAL) and a single-arm trial of selinexor and low-dose dexamethasone in patients with multiple myeloma (STORM). Additional Phase 1 and Phase 2 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 KPT-8602

KPT-8602 is a second generation oral SINETM compound. KPT-8602 functions by binding to and inhibiting the nuclear export protein XPO1 (also called CRM1), leading to the accumulation of tumor suppressor proteins in the cell nucleus. KPT-8602 has demonstrated minimal brain penetration in animals, which has been associated with reduced toxicities in preclinical studies while maintaining potent anti-tumor effects.

#### About KPT-9274

KPT-9274 is a first-in-class orally bioavailable small molecule that is a non-competitive dual modulator of PAK4 and NAMPT. Co-inhibition of these targets leads to synergistic anti-tumor effects through energy depletion, inhibition of DNA repair, cell cycle arrest, inhibition of proliferation, and ultimately apoptosis.

## 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 was founded by Dr. Sharon Shacham and is located in Newton, Massachusetts. 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), KPT-8602, KPT-9274 or any other drug candidate that Karyopharm is developing 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; 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, 2015, which is on file with the Securities and Exchange Commission (SEC) as of March 15, 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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