

Karyopharm Announces the Presentation of New Data at the 2017 American Association for Cancer Research Annual Meeting

Late-Breaking Presentation Highlighting Interim Phase 2b Selinexor Data in Patients with Relapsed or Refractory DLBCL (SADAL Study) Presentations Include Eleven Preclinical Abstracts Describing Key Scientific Findings across the Company's Oncology Programs Overview of Key Selinexor Myeloma Data Also Featured at the 16th International Myeloma Workshop

NEWTON, Mass., March 01, 2017 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a clinical-stage pharmaceutical company, today announced that 12 abstracts describing the Company's product candidates in development for hematological and solid tumor malignancies have been selected for presentation at the 2017 Annual Meeting of the American Association for Cancer Research (AACR) taking place April 1-5, 2017 in Washington, DC. The abstracts, which represent both company- and investigator-sponsored studies, describe data related to Karyopharm's lead product candidate, selinexor (KPT-330), an oral Selective Inhibitor of Nuclear Export / SINE™ compound, as well as two of its promising Phase 1 oncology programs, KPT-8602, a second-generation oral SINE compound, and KPT-9274, a first-in-class oral dual inhibitor of PAK4 and NAMPT.

"The ongoing randomized Phase 2b SADAL study, which was initiated based on encouraging Phase 1 data in patients with diffuse large B-cell lymphoma (DLBCL), was designed to evaluate the overall response rate of single-agent oral selinexor in patients with relapsed or refractory DLBCL," said Sharon Shacham, PhD, MBA, President and Chief Scientific Officer of Karyopharm. "We look forward to presenting interim results from this important trial at AACR this year."

Karyopharm is also presenting an overview of selinexor myeloma data at the 16th International Myeloma Workshop (IMW) held March 1-4, 2017 in New Delhi, India. In an oral presentation, titled "Oral Selinexor Shows Single Agent Activity Enhanced with PI or IMiD Combinations in Refractory Multiple Myeloma," (Abstract #234) Sagar Lonial, MD, FACP, Professor and Chair, Hematology and Medical Oncology, Emory University, provided an overview of clinical data demonstrating selinexor's activity in combination with proteasome inhibitors (PIs) and immunomodulatory drugs (IMiDs) for the treatment of relapsed or refractory multiple myeloma. The 2017 IMW is a prestigious biannual event where myeloma experts from around the world gather to discuss basic, preclinical and clinical aspects in the biology and treatment of multiple myeloma.

Late-Breaking Poster Presentations 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

Title: KPT-9274 inhibits cellular NAD and synergizes with doxorubicin to treat dogs with lymphoma

Presenter: Cheryl London, Tufts University

Poster Board #: 16

Session: Late-Breaking Research: Experimental and Molecular Therapeutics 2

Location: Convention Center, Halls A-C, Poster Section 34

Date and Time: Wednesday, April 5, 2017 8:00 AM - 12:00 PM

Other Presentations at AACR 2017:

Title: [Selinexor or KPT-8602 mediated XPO1 inhibition synergizes with dexamethasone to repress convergent pathways in the mTORC1 signaling network and drive cell death in multiple myeloma](#)

Presenter: Christian Argueta, Karyopharm Therapeutics Inc.

Poster Board #: 15

Session: Molecular and Cellular Biology/Genetics — Cell Growth Signaling Pathways 1

Location: Convention Center, Halls A-C, Poster Section 14

Date and Time: Sunday, April 2, 2017 1:00 PM - 5:00 PM

Title: [Novel role of XPO1 in regulating microRNAs related to pancreatic ductal adenocarcinoma invasion and metastasis](#)

Presenter: Asfar Azmi, Wayne State University

Poster Board #: 5

Session: Molecular and Cellular Biology/Genetics — MicroRNA Regulation of Cancer Biology 1

Location: Convention Center, Halls A-C, Poster Section 19

Date and Time: Sunday, April 2, 2017 1:00 PM - 5:00 PM

Title: [Synergistic effects of the XPO1 inhibitor selinexor with proteasome inhibitors in pediatric high-grade glioma and diffuse intrinsic pontine glioma](#)

Presenter: John DeSisto, University of Colorado Denver

Poster Board #: 18

Session: Tumor Biology: Pediatric Cancer 1: Biomarkers, Preclinical Models, and New Targets

Location: Convention Center, Halls A-C, Poster Section 42

Date and Time: Monday, April 3, 2017 8:00 AM - 12:00 PM

Title: [Anti-tumor activity of selinexor is enhanced by palbociclib in preclinical models of HER2+ breast cancer](#)

Presenter: Hua Chang, Karyopharm Therapeutics Inc.

Poster Board #: 12

Session: Experimental and Molecular Therapeutics — Combination Therapy 1

Location: Convention Center, Halls A-C, Poster Section 2

Date and Time: Monday, April 3, 2017 8:00 AM - 12:00 PM

Title: [Disruption of nuclear export with selinexor or KPT-8602 reduces androgen receptor expression and leads to potent anti-tumor activity in preclinical models of androgen-independent prostate cancer](#)

Presenter: Christian Argueta, Karyopharm Therapeutics Inc.

Poster Board #: 13

Session: Endocrinology — Prostate Cancer Biology and Therapy

Location: Convention Center, Halls A-C, Poster Section 25

Date and Time: Monday, April 3, 2017 8:00 AM - 12:00 PM

Title: [p21 activated kinase 4 \(PAK4\) as a novel therapeutic target for non-Hodgkin's lymphoma](#)

Presenter: Asfar Azmi, Wayne State University

Poster Board #: 9

Session: Molecular and Cellular Biology/Genetics — Cell Growth Signaling Pathways 4

Location: Convention Center, Halls A-C, Poster Section 14

Date and Time: Monday, April 3, 2017 8:00 AM - 12:00 PM

Title: [Nuclear export of E2F7 in squamous cell carcinoma is an actionable event that reverses resistance to anthracyclines](#)

Presenter: Alba Natalia Saenz Ponce, University of Queensland, Brisbane, Australia

Poster Board #: 28

Session: Experimental and Molecular Therapeutics: Reversal of Drug Resistance

Location: Convention Center, Halls A-C, Poster Section 6

Date and Time: Monday, April 3, 2017 8:00 AM - 12:00 PM

Title: [Exportin-1 \(XPO1\) is a novel therapeutic biomarker for patients with neuroblastoma](#)

Presenter: Basia Galinski, Albert Einstein College of Medicine

Poster Board #: 10

Session: Pediatric Cancer 1: Biomarkers, Preclinical Models, and New Targets

Location: Convention Center, Halls AC, Poster Section 42

Date and Time: Monday, April 3, 2017 8:00 AM - 12:00 PM

Title: [Combined targeting of estrogen receptor alpha and nuclear transport pathways remodel metabolic pathways to induce apoptosis and overcome tamoxifen resistance](#)

Presenter: Eylem Kulkoyluoglu-Cotul, University of Illinois Urbana-Champaign

Poster Board #: 14

Session: Endocrinology: Nuclear Receptors and Endocrine Oncology Therapies

Location: Convention Center, Halls A-C, Poster Section 25

Date and Time: Tuesday, April 4, 2017 8:00 AM - 12:00 PM

Title: [Selinexor synergizes with DNA damaging agents through down-regulation of key DNA damage response genes](#)

Presenter: Trinayan Kashyap, Karyopharm Therapeutics Inc.

Poster Board #: 26

Session: Experimental and Molecular Therapeutics — New Targets and New Drugs

Location: Convention Center, Halls A-C, Poster Section 5

Date and Time: Tuesday, April 4, 2017 1:00 PM - 5:00 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. 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. The latest 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), KPT-8602 and KPT-9274, 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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