

Karyopharm to Present Data on Oncology Pipeline at American Society of Hematology 2015 Annual Meeting

NEWTON, Mass., Nov. 5, 2015 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a clinical-stage pharmaceutical company, today announced that 17 abstracts, including 5 oral presentations and 12 posters describing the activity of its oncology pipeline for the treatment of hematologic malignancies, have been selected for presentation at the 2015 American Society of Hematology (ASH) annual meeting taking place December 5-8, 2015 in Orlando, Florida. The abstracts, which represent both company and investigator-sponsored clinical and preclinical studies, describe data related to selinexor, Karyopharm's first-in-class, oral Selective Inhibitor of Nuclear Export (SINE™) compound that inhibits exportin 1 (XPO1), as well as preclinical data on other pipeline programs, including a second generation SINE compound, KPT-8602, and PAK4 Allosteric Modulators (PAMs). Data from these abstracts will be updated in presentations and posters at ASH.

"The breadth of data to be presented on our oncology pipeline at ASH demonstrates the increasing level of effort we and our collaborators are deploying to leverage the mechanism for XPO1 inhibition across a wide range of hematologic malignancies," said Sharon Shacham, PhD, MBA, President and Chief Scientific Officer of Karyopharm. "Selinexor continues to demonstrate encouraging activity and tolerability in combination with other anti-cancer agents and our discovery efforts have generated new oncology pipeline candidates, including KPT-8602, a second generation SINE compound, and PAK4 Allosteric Modulators. We look forward to sharing this ample and growing body of data on our oncology pipeline at ASH."

Selinexor Clinical Presentations:

Title (oral): Safety, Efficacy, and Determination of the Recommended Phase 2 Dose for the Oral Selective Inhibitor of Nuclear Export (SINE) Selinexor (KPT-330)

Author: Christine Chen, Princess Margaret Cancer Center

Abstract: 258

Session: 623. Lymphoma: Chemotherapy, excluding Pre-Clinical Models: NHL - New Drugs

Date/Time: Sun, Dec 6, 12:00 - 1:30 pm

Title (poster): Phase 1 MMRC Trial of Selinexor, Carfilzomib (CFZ), and Dexamethasone (DEX) in Relapsed and Relapsed/Refractory Multiple Myeloma (RRMM)

Author: Andrzej J. Jakubowiak, The University of Chicago

Abstract: 4223

Session: 653. Myeloma: Therapy, excluding Transplantation: Poster III

Date/Time: Mon, Dec 7, 6:00 - 8:00 pm

Title (poster): Selinexor, ARA-C and Idarubicin: An Effective and Tolerable Combination in Patients with Relapsed/Refractory AML: A Multicenter Phase II Study

Author: Walter Fiedler, MD, University Medical Center Hamburg-Eppendorf, Germany

Abstract: 3789

Session: 616. Acute Myeloid Leukemia: Novel Therapy, excluding Transplantation: Poster III

Date/Time: Mon, Dec 7, 6:00 - 8:00 pm

Title (poster): Phase I Study of Selinexor, a Selective Inhibitor of Nuclear Export, in Combination with Fludarabine and Cytarabine in Pediatric Patients with Relapsed or Refractory AML

Author: Jeffrey E Rubnitz, MD, St. Jude Children's Research Hospital

Abstract: 1345

Session: 616. Acute Myeloid Leukemia: Novel Therapy, excluding Transplantation: Poster I

Date/Time: Sat, Dec 5, 5:30 - 7:30 pm

Selinexor and SINE Preclinical Presentations:

Title (oral): Recurrent mutations of the exportin 1 gene (XPO1) in primary mediastinal B-cell lymphoma: a LYSA study

Author: Fabrice Jardin, MD-PhD, INSERM, France

Abstract: 129

Session: 622. Non-Hodgkin's Lymphoma

Date/Time: Sat, Dec 5, 4:00 - 5:30 pm (4:30 pm)

Title (oral): The Gain of the Short Arm of Chromosome 2 (2p+) Induces XPO1 Overexpression and Drug Resistance in Chronic Lymphocytic Leukemia

Author: Adrien Cosson, INSERM, France
Abstract: 492
Session: 641. CLL
Date/Time: Mon, Dec 7, 7:00 - 8:30 am (8:15 am)

Title (poster): Selective Inhibitors of Nuclear Export (SINE) Compounds Suppress both HIV Replication and AIDS Related Lymphoma

Author: Dirk Daelemans, Rega Institute for Medical Research, Belgium
Abstract: 2751
Session: 625. Lymphoma: Pre-Clinical - Chemotherapy and Biologic Agents: Poster II
Date/Time: Sun, Dec 6, 6:00 - 8:00 pm

Title (poster): Selinexor Is an Effective Cancer Treatment in Hypoxic Conditions and Synergizes with Proteasome Inhibitors to Treat Drug Resistant Multiple Myeloma

Author: Barbara Muz, Washington University in Saint Louis School of Medicine
Abstract: 3017
Session: 652. Myeloma: Pathophysiology and Pre-Clinical Studies, excluding Therapy: Poster II
Date/Time: Sun, Dec 6, 6:00 - 8:00 pm

Title (poster): Combination Therapy of Selinexor with Bortezomib or Carfilzomib Overcomes Drug Resistance to Proteasome Inhibitors (PI) in Human Multiple Myeloma

Author: Joel G. Turner, H. Lee Moffitt Cancer Center
Abstract: 3048
Session: 653. Myeloma: Therapy, excluding Transplantation: Poster II
Date/Time: Sun, Dec 6, 6:00 - 8:00 pm

Title (poster): Development of a Pharmacodynamic Assay for XPO1 Occupancy Using Fluorescence Cross Correlation Spectroscopy (FCCS)

Author: Marsha L. Crochiere, Karyopharm Therapeutics
Abstract: 2487
Session: 605. Molecular Pharmacology, Drug Resistance - Lymphoid and Other Diseases: Poster II
Date/Time: Sun, Dec 6, 6:00 - 8:00 pm

Title (poster): Selinexor, a Selective Inhibitor of Nuclear Export (SINE) Compound, Shows Synergistic Anti-Tumor Activity in Combination with Dexamethasone Characterized by Specific Pattern of Gene Expression in Multiple Myeloma (MM)

Author: Trinayan Kashyap, Karyopharm Therapeutics
Abstract: 3683
Session: 604. Molecular Pharmacology and Drug Resistance in Myeloid Diseases: Poster III
Date/Time: Mon, Dec 7, 6:00 - 8:00 pm

Title (poster): XPO1 (Exportin-1) Is a Major Regulator of Human Erythroid Differentiation. Potential Clinical Applications to Decrease Ineffective Erythropoiesis of Beta-Thalassemia

Author: Guillem Flavia, IMAGINE INSTITUTE, France
Abstract: 2368
Session: 501. Hematopoietic Stem and Progenitor Biology: Poster II
Date/Time: Sun, Dec 6, 6:00 - 8:00 pm

KPT-8602 Preclinical Presentations

Title (oral): Nuclear Export Inhibitor KPT-8602 Is Highly Active against Leukemic Blasts and Leukemia-Initiating Cells in Patient-Derived Xenograft Models of AML

Author: Julia Etchin, PhD, Dana Farber Cancer Institute
Abstract: 326
Session: 616. Acute Myeloid Leukemia: Novel Therapy, excluding Transplantation: New Drugs
Date/Time: Sun, Dec 6, 4:30 - 6:00 pm (4:45pm)

Title (oral): Next Generation XPO1 Inhibitor Shows Improved Efficacy and In Vivo Tolerability in Hematologic Malignancies

Author: Zachary A. Hing, The Ohio State University

Abstract: 317

Session: 605. Molecular Pharmacology, Drug Resistance - Lymphoid and Other Diseases: Novel Targets and Therapeutics

Date/Time: Sun, Dec 6, 4:30 - 6:00 pm (5:30pm)

Title (poster): Next Generation XPO1 Inhibitor KPT-8602 for the Treatment of Drug-Resistant Multiple Myeloma

Author: Joel G. Turner, H. Lee Moffitt Cancer Center and Research Institute

Abstract: 1818

Session: 652. Myeloma: Pathophysiology and Pre-Clinical Studies, excluding Therapy: Poster I

Date/Time: Sat, Dec 5, 5:30 - 7:30 pm

PAK4 Allosteric Modulators Preclinical Presentations

Title (poster): In vitro and in vivo anti-leukemic effects of PAK4 Allosteric Modulators in Acute Myeloid Leukemia: Promising Results Justifying Further Development

Author: Shaneice Mitchell, The Ohio State University

Abstract: 2471

Session: 604. Molecular Pharmacology and Drug Resistance in Myeloid Diseases: Poster II

Date/Time: Sun, Dec 6, 6:00 - 8:00 pm

Title (poster): Dissecting Signaling Network Responses to PAK4 Allosteric Modulators (PAMs) in Cell Subsets within Primary Human Acute Myeloid Leukemia Samples

Author: Paul Brent Ferrell Jr., MD, Vanderbilt University Medical Center

Abstract: 3686

Session: 604. Molecular Pharmacology and Drug Resistance in Myeloid Diseases: Poster III

Date/Time: Mon, Dec 7, 6:00 - 8:00 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 1,200 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. Karyopharm has initiated four later-phase clinical trials of selinexor, 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) and a single-arm trial of selinexor and low-dose dexamethasone in patients with multiple myeloma (STORM). In solid tumors, Karyopharm plans to initiate a randomized, placebo-controlled Phase 2/3 trial of selinexor to treat liposarcoma during the fourth quarter of 2015. 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.

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 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 activity against a variety of different human cancers, SINE™ compounds have also shown biological activity in models of cancer, 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.

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) and KPT-8602, or any other drug candidate, including PAK4 Allosteric Modulators 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 Quarterly Report on Form 10-Q for the quarter ended June 30, 2015, which is on file with the Securities and Exchange Commission (SEC) as of August 10, 2015, 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.

CONTACT: Justin Renz

(617) 658-0574

jrenz@karyopharm.com

Gina Nugent

(617) 460-3579

nugentcomm@aol.com

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