Karyopharm Therapeutics Announces Award of Grant From National Multiple Sclerosis Society to Further Develop Selective Inhibitors of Nuclear Export (SINE) Compounds

NATICK, Mass., Jan. 8, 2014 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), 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, today announced that it has been awarded a grant from the National Multiple Sclerosis (MS) Society through its affiliate, Fast Forward, to support research on the potential of SINE compounds in inflammatory models of disease, including MS. Finding ways to restore and protect the damaged nervous system is a key priority of the National MS Society's No Opportunity Wasted (NOW) research campaign to ultimately eradicate MS.

"We are very excited about our continuing collaboration with the National MS Society, and with Mount Sinai with Dr. Patrizia Casaccia, who has dedicated herself to advancing research in MS and other important diseases," said Karyopharm Founder, Chief Scientific Officer, and President, Sharon Shacham, Ph.D., M.B.A. "We believe SINE compounds may offer significant therapeutic benefits in a range of indications and look forward to exploring the potential application of SINE compounds in inflammatory diseases, including MS."

SINE compounds inhibit Exportin-1 (XPO1 or CRM1), which mediates the export of approximately 220 different mammalian cargo proteins. In MS, toxic factors accumulate in the brain and spinal cord that may attack or destroy the myelin coating on a neuron's axon causing electrical signals to other nerve cells, muscles, and cells throughout the body to slow, thus leading to neurodegenerative symptoms. Preclinical data suggest that SINE compounds may reduce inflammation and protect against these toxic factors in neurons, which could potentially reduce the perpetuation and progression of MS.

Dr. Patrizia Casaccia, M.D., Ph.D., Professor in the Departments of Neuroscience and Genetics and Genomics at Icahn School of Medicine at Mount Sinai, is the academic lead on the study. Her laboratory first identified nuclear export as a potentially important mechanism in neurodegeneration and conducted preliminary experiments using mouse models. Her experiments suggest that oral administration of a novel SINE compound to mice with ongoing paralysis of the tail and hindlimb may improve their walking ability. Dr. Casaccia will continue this research, in conjunction with Karyopharm, to further study the mechanism of action and safety profile of SINE compounds in inflammatory models and to gather data to potentially select a SINE compound as a candidate for early-stage clinical trials in human patients with MS.

Dr. Casaccia underscored the potential of SINE compounds as a novel treatment for MS. "What's unique about this work is that these oral SINE compounds target and prevent nuclear export, which is central to the neurodegenerative phase of MS. We currently have no drugs in MS that work by this mechanism," she said.

About Karyopharm

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 blocking the XPO1, preventing the export of various proteins out of the nucleus. SINE compounds have shown biological activity in models of cancer, autoimmune disease, certain viruses, and wound-healing. Karyopharm was founded by Dr. Sharon Shacham and is located in Natick, Massachusetts.

About Multiple Sclerosis

Multiple sclerosis, an unpredictable, often disabling disease of the central nervous system, interrupts the flow of information within the brain, and between the brain and body.

About the National MS Society and Fast Forward, LLC

The National MS Society mobilizes people and resources to drive research for a cure and to address the challenges of everyone affected by MS. To fulfill this mission, the Society funds cutting-edge research, drives

change through advocacy, facilitates professional education, collaborates with MS organizations around the world, and provides programs and services designed to help people with MS and their families move their lives forward. In 2013 alone, through its home office and 50-state network of chapters, the Society invested $48.3 million to support 380 new and ongoing research projects around the world. The Society is dedicated to achieving a world free of MS.

Fast Forward, LLC was established by the National Multiple Sclerosis Society as part of a comprehensive approach to MS research and treatment, focusing on accelerating commercial development of promising research discoveries. Through Fast Forward, the Society connects university-based MS research with private-sector drug development and funds small biotechnology/pharmaceutical companies to develop innovative new MS therapies and repurpose FDA-approved drugs as new treatments for MS.

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 SINE compounds, 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), or any other drug candidate 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 September 30, 2013, which is on file with the Securities and Exchange Commission (SEC), 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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