Karyopharm and Collaborators Awarded Grant for ALS Research

- Target ALS Consortium Grants $900,000 in Research Funding for KPT-350 in ALS -
- Fifth Grant Awarded in Support of KPT-350 as a Potential ALS Treatment Option -

NEWTON, Mass., Dec. 15, 2016 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a clinical-stage pharmaceutical company, today announced that Target ALS Foundation, a non-profit organization with the overall goal of accelerating development of new treatments for amyotrophic lateral sclerosis (ALS), has granted $900,000 in research funding over a two-year period to support preclinical studies of KPT-350 in ALS. The project, led by Karyopharm in collaboration with researchers from Johns Hopkins University and the University of Florida, will study KPT-350 in preclinical models and seek to develop an oral suspension formulation to dose patients who cannot swallow tablets. This grant from Target ALS represents the fifth grant awarded to Karyopharm in support of ALS research for KPT-350.

"We are honored to receive this grant which was awarded based on a long and successful collaboration with researchers at Johns Hopkins University, including Jeffrey Rothstein, MD, PhD and Thomas Lloyd MD, PhD, and Laura Ranun, PhD from the University of Florida. There is an immediate need for therapies focused on reducing and preventing disease progression in patients with ALS, a debilitating, incurable and uniformly fatal disease," said Sharon Shacham, PhD, MBA, President and Chief Scientific Officer of Karyopharm. "We have observed striking neuroprotective and anti-inflammatory effects of KPT-350 in preclinical disease models across a variety of neuroinflammatory disorders, suggesting that inhibition of XPO1 activity in the CNS by KPT-350 represents a novel mechanism that warrants further evaluation in neurological indications such as ALS. We are striving to bring this novel oral therapy into the clinic as soon as possible."

About ALS

Amyotrophic lateral sclerosis (ALS), sometimes called Lou Gehrig's disease, is a rapidly progressive, invariably fatal neurological disease that attacks the nerve cells responsible for controlling voluntary muscles. The disease belongs to a group of disorders known as motor neuron diseases, which are characterized by the gradual degeneration and death of motor neurons.

ALS causes weakness with a wide range of disabilities. Eventually, all muscles under voluntary control are affected, and individuals lose their strength and the ability to move their arms, legs, and body. When muscles in the diaphragm and chest wall fail, people lose the ability to breathe without ventilator support. Most people with ALS die from respiratory failure, usually within three to five years from the onset of symptoms. However, about 10 percent of those with ALS survive for 10 or more years.

About KPT-350

KPT-350, an oral SINETM compound, is an investigational new drug application-ready oral compound with preclinical data supporting potential efficacy in a number of neuroinflammatory conditions, namely ALS, multiple sclerosis (MS), Huntington's disease (HD) and traumatic brain injury (TBI). KPT-350 inhibits the activity of Exportin 1 (XPO1) and efficiently crosses the blood-brain barrier. XPO1 is a key mediator of the nuclear export of proteins, many of which control transcription of downstream neuroprotectant genes including potential therapeutic targets for ALS. In a preliminary in vivo study in rats, KPT-350 ameliorated the ALS-like disease state that was induced by gene transfer of TDP-43. KPT-350 demonstrated a neuroprotective effect in primary neuronal models of ALS and frontotemporal degeneration (FTD), and restored nuclear trafficking defects and ocular neurodegeneration in a Drosophila C9orf72-mediated model of ALS. KPT-350 enhances additional neuroprotective processes and preserves blood brain barrier integrity. XPO1 inhibition also leads to potent, multifaceted inhibition of the inflammatory mediator nuclear factor kappa-B, or NF-kB, a protein that plays very important roles in many types of inflammation that can accelerate neuronal death. Preclinical data, generated mainly by Karyopharm's academic collaborators, has shown efficacy of orally-administered KPT-350 in animal models of ALS, MS and TBI.

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. 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 drug candidates, including SINE™ compounds like KPT-350, will successfully complete necessary preclinical studies to permit healthy human volunteers or patients to be treated or that, if commenced, clinical development phases 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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