

# Karyopharm Receives NIAID Grant to Advance Development of KPT-350 for the Treatment of Lupus

NEWTON, Mass., March 31, 2016 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a clinical-stage pharmaceutical company, today announced that it has been awarded a grant from the National Institute of Allergy and Infectious Disease (NIAID) to advance development of KPT-350, an oral Selective Inhibitor of Nuclear Export / SINE™ compound for the treatment of inflammatory, autoimmune and neurological diseases. The \$225,000 grant will be used to conduct further preclinical studies of KPT-350 for the treatment of systemic lupus erythematosus, or lupus, a chronic, autoimmune disease that causes the body to create autoantibodies that attack and destroy healthy tissue, causing inflammation, pain, and damage in various parts of the body. These studies will be conducted in collaboration with Dr. Jenifer Anolik, MD, PhD, Associate Professor of Medicine at the University of Rochester.

"XPO1 inhibition is known to generate potent, multifaceted inhibition of the inflammatory mediator NF-κB. KPT-350 inhibits XPO1 leading to increases in the levels of several natural, cellular inhibitors of NF-κB, thus blocking its inflammatory activity. This results in an increase in anti-inflammatory and neuroprotective responses leading to reduced autoimmune disease activity," said Sharon Shacham, PhD, MBA, President and Chief Scientific Officer of Karyopharm. "With this funding support from NIAID, we are eager to advance the development of KPT-350 with Dr. Anolik to further evaluate its potential activity in chronic and painful inflammatory and autoimmune diseases such as lupus."

KPT-350 is an investigational new drug application-ready oral compound with preclinical data supporting potential efficacy in a number of neurological, autoimmune and inflammatory conditions. Preclinical data, generated mainly by Karyopharm's academic collaborators, has shown efficacy of orally-administered KPT-350 in animal models of lupus, and other autoimmune, inflammatory and neurological conditions.

This research will be supported by the National Institute of Allergy And Infectious Diseases of the National Institutes of Health under Award Number R43AI124949. The studies are solely the responsibility of the researchers and do not necessarily represent the official views of the National Institutes of Health.

## About KPT-350

KPT-350 is an oral SINE™ compound being developed for the treatment of inflammatory and autoimmune diseases including lupus. XPO1 inhibition leads to potent, multifaceted inhibition of the inflammatory mediator nuclear factor kappa-light-chain-enhancer of activated B cells, or NF-κB, a protein that plays very important roles in many types of inflammation. KPT-350 has additional important activities such as activation of proteins leading to anti-oxidant and neuroprotective properties. Karyopharm is evaluating KPT-350, in additional inflammatory, autoimmune and neurological disease models.

## 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 to 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 virus infections 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 KPT-350 or any of Karyopharm's other SINE™ compounds, including selinexor (KPT-330), 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.

Contact:

Justin Renz

(617) 658-0574

[jrenz@karyopharm.com](mailto:jrenz@karyopharm.com)

Gina Nugent

(617) 460-3579

[nugentcomm@aol.com](mailto:nugentcomm@aol.com)

Source: Karyopharm Therapeutics

News Provided by Acquire Media



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