

Phase 2 Study of Selinexor (KPT-330) Initiated by Karyopharm in Patients With Recurrent Glioblastoma After Failure of Radiation and Temozolomide (KING Study)

NATICK, Mass., April 29, 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 the initiation of a Phase 2 trial of its novel, oral Selective Inhibitor of Nuclear Export (SINE) compound Selinexor (KPT-330) in patients with glioblastoma following treatment with radiation and temozolomide. The study, referred to as the KING study, is being run by Drs. Morten Mau-Sørensen at the Rigshospitalet in Copenhagen, Denmark, Andrew Lassman at Columbia University, New York, and Patrick Wen, Dana Farber Cancer Institute, Boston, Massachusetts.

Eligible patients have disease that has recurred after prior treatment with radiation therapy and temozolomide and may undergo surgery as required. The primary goal of the study is to determine the anti-tumor activity of single agent Selinexor in up to 30 patients with relapsed glioblastoma (grade 4 glioma), as well as to document brain penetration of Selinexor and determine tolerability in this population. Full description of the study is available at www.clinicaltrials.gov (NCT01986348).

Selinexor is a covalent inhibitor of the nuclear export protein XPO1 that forces the accumulation and activation of multiple tumor suppressor proteins in the nucleus. This leads to induction of apoptosis in neoplastic cells, while largely sparing normal cells. Preclinical results have shown that Selinexor kills glioblastoma neurospheres in culture, crosses the blood brain barrier of animals and has activity against glioblastoma xenografts and primagrafts with various genetic lesions.

Dr. Mau-Sørensen stated, "We are very pleased to initiate this multi-center study of oral Selinexor in patients with recurrent glioblastoma. This is a very difficult-to-treat patient population with limited options. Because Selinexor works by a completely novel mechanism, crosses the blood brain barrier and has shown activity in preclinical models of glioblastoma, we look forward to the results of this study."

"This study recognizes our commitment to the treatment of patients with recurrent brain tumors, and we hope will set a foundation for the combination of Selinexor with other treatment modalities in the future," commented Dr. Sharon Shacham, founder, President and CSO of Karyopharm. "In addition, this study could provide a basis for the evaluation of other types of central nervous system tumors, as well as patients with brain metastases associated with non-brain cancers."

About Selinexor

Selinexor (KPT-330) is a first-in-class, oral Selective Inhibitor of Nuclear Export (SINE) compound. Selinexor functions by binding with the nuclear export protein XPO1 (also called CRM1), leading to the accumulation of tumor suppressor proteins in the cell nucleus, which subsequently reinitiates and amplifies their tumor suppressor function. This is believed to lead to the selective induction of apoptosis in cancer cells, while largely sparing normal cells. Over 300 patients have been treated with Selinexor in Phase 1 and Phase 2 trials in advanced hematologic malignancies and solid tumors. Additional Phase 1 and Phase 2 studies are ongoing or currently planned and three registration-directed clinical trials in hematological indications are expected to begin enrollment during 2014. 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. 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.

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), 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, 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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