Karyopharm Presents Clinical Data for Selinexor (KPT-330) in Patients With Recurrent Glioblastoma and Advanced Sarcomas at 2015 ASCO Annual Meeting

NEWTON, Mass., June 1, 2015 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a clinical-stage pharmaceutical company, today announced the presentation of positive clinical data for its lead product candidate, Selinexor (KPT-330), a first-in-class, oral Selective Inhibitor of Nuclear Export / SINE™ compound, at the 2015 American Society of Clinical Oncology (ASCO) Annual Meeting. In an ongoing Phase 2 clinical trial, single-agent oral selinexor demonstrated anti-tumor activity in patients with recurrent glioblastoma, including brain penetration at clinically relevant drug levels, with a 13% overall response rate and a 38% disease control rate. In an ongoing Phase 1b clinical study of single agent oral selinexor in patients with advanced sarcomas including liposarcoma, durable activity, including longer progression free survival than last prior regimen, was demonstrated.

"These clinical data extend clinically relevant levels of selinexor in brain tumors, with tolerable and durable anti-cancer activity and disease control in recurrent glioblastoma, as well as durable stable disease in liposarcoma and other sarcomas. These data provide further support for our broad solid tumor development plans for selinexor as a single-agent and in combination with approved or experimental therapies," said Sharon Shacham, PhD, MBA, President and Chief Scientific Officer of Karyopharm.

In a poster presented on Monday, June 1, 2015, entitled, "A phase 2 study on efficacy, safety and intratumoral pharmacokinetics of oral selinexor (KPT-330) in patients with recurrent glioblastoma (GBM)," Dr. Ullrik Lassen of Rigshospitalet and colleagues described data from an ongoing Phase 2 study of single-agent selinexor in patients with glioblastoma that recurred after temozolomide and radiation therapy, including brain penetration at clinically relevant levels, leading to durable anti-cancer activity and disease control of up to 6 months. All data are as of May 10, 2015.

- Selinexor dosed twice weekly at 50 mg/m2 demonstrated anti-tumor activity with 13% ORR and 38% DCR in 16 surgically ineligible patients with glioblastoma that had been pretreated with temozolomide and radiation. Response data across these 16 patients were as follows:

N	DCR	PR	SD	PD
16	6 (38%)	2 (13%)	4 (25%)	10 (62%)

Responses allocated according to the Response Assessment in Neuro-Oncology (RANO). DCR=Disease Control Rate (PR+SD), PR=Partial Response, SD=Stable Disease, PD=Progressive Disease

- Selinexor reaches concentrations in glioblastoma tumors that are active in vitro against patient-derived glioblastoma cells.
- The most common adverse events were thrombocytopenia, fatigue, anorexia, and hyponatremia.

In a poster presented on Sunday, May 31, 2015, entitled, "A phase 1b study with selinexor, a first in class selective inhibitor of nuclear export (SINE) in patients with advanced sarcomas: An efficacy analysis," Dr. Mrinal Gounder of Memorial Sloan Kettering Cancer Center and colleagues described the activity of single-agent selinexor in patients with advanced sarcomas in which durable stable-disease (including tumor shrinkage) was observed. All data are as of May 10, 2015.

- In 45 evaluable patients receiving single-agent selinexor dosed twice weekly, 27 patients (60%) across a variety of sarcoma types achieved stable disease.

Sarcoma Type	N	SD (%)	PD (%)
Liposarcoma	18	14 (78%)	4 (22%)
Leiomyosarcoma	8	5 (63%)	3 (37%)
Others	19	8 (42%)	11 (58%)
Total	45	27 (60%)	18 (40%)

Responses adjudicated according to the Response Evaluation Criteria in Solid Tumors (RECIST v1.1).
SD=Stable Disease, PD=Progressive Disease

- Median progression free survival for selinexor was 136 days compared with last prior regimen of 54 days in 11 liposarcoma patients with known time to progression on last prior regimen.
- Selinexor was generally well tolerated with supportive care for anorexia and nausea.
- Selinexor was dosed twice-weekly at either 30 mg/m2 (~50 mg), 50 mg/m2 (~80 mg) or 60 mg flat dose.
- On-treatment biopsies demonstrated the pharmacological activity of selinexor based upon decreased tumor cell numbers, reduced proliferative rates and increased replacement of tumor with stromal tissue compared with pre-treatment biopsies.

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,

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 900 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 three registration-directed clinical trials of selinexor, including one in older patients with acute myeloid leukemia (SOPRA), one in patients with Richter's transformation (SIRRT) and one in patients with diffuse large B-cell lymphoma (SADAL). A single-arm trial of selinexor in patients with multiple myeloma (STORM) that is also intended to be registration-directed was initiated in May 2015. In solid tumors, Karyopharm plans to initiate a registration-directed trial of selinexor to treat liposarcoma during the second half 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) or any PAK4 inhibitor, 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 Quarterly Report on Form 10-Q for the quarter ended March 31, 2015, which is on file with the Securities and Exchange Commission (SEC) as of May 11, 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.

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