Karyopharm Presents Updated Phase 2b STORM Data at the American Society of Hematology 2016 Annual Meeting

- Data Continue to Reinforce the Efficacy and Safety of Selinexor in Patients with Heavily Pretreated Refractory Multiple Myeloma -
- Company to Host Dinner Reception and Webcast Event with Interactive Expert Panel Discussion on Monday, December 5, 2016 at 8:15 p.m. PT -

NEWTON, Mass., Dec. 04, 2016 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a clinical-stage pharmaceutical company, today announced updated results from its Phase 2b STORM study of selinexor (KPT-330), including robust rates and duration of response, compelling overall survival and a favorable safety profile, in patients with heavily pretreated refractory multiple myeloma (MM) at the American Society of Hematology (ASH) 2016 annual meeting held December 3-6, 2016 in San Diego. Selinexor is the Company's lead, novel, oral Selective Inhibitor of Nuclear Export (SINE™) compound, in development for the treatment of a variety of malignancies, including MM and acute myeloid leukemia (AML).

"The data presented today further support the rationale for selinexor as a promising new treatment for patients with refractory myeloma with no clearly beneficial treatment options," said Sharon Shacham, PhD, MBA, President and Chief Scientific Officer of Karyopharm. "Based on the exciting STORM data and the existing unmet medical need, we have expanded the study to include additional patients with penta-refractory myeloma and expect to report top-line data from this study in early 2018."

Updated Phase 2b STORM Clinical Data in Refractory Multiple Myeloma

In an oral presentation titled, "Selinexor and Low Dose Dexamethasone in Patients with Lenalidomide, Pomalidomide, Bortezomib, Carfilzomib and Anti-CD38 Ab Refractory MM STORM Study," Dan T. Vogl, MD, MSCE, Assistant Professor of Medicine, Perelman School of Medicine, University of Pennsylvania, presented updated clinical data from the ongoing Phase 2b STORM study, a single-arm clinical trial evaluating selinexor in combination with low-dose dexamethasone in patients with quad-refractory or penta-refractory myeloma. Patients with quad-refractory disease have previously received two proteasome inhibitors (PIs) (bortezomib (Velcade®) and carfilzomib (Kyprolis®)) and two immunomodulatory drugs (IMiDs) (lenalidomide (Revlimid®) and pomalidomide (Pomalyst®)), and their disease is refractory to at least one PI, at least one IMiD, and has progressed following their most recent therapy. Patients with penta-refractory myeloma have quad-refractory disease that is also refractory to an anti-CD38 monoclonal antibody, such as daratumumab (Darzalex®) or isatuximab.

Phase 2b STORM Efficacy

Category	N1	ORR (%)	CBR (%)	VGPR (%)	PR (%)	MR (%)	SD (%)	PD (%)	NE (%)
Overall	78	16 (21%)	26 (33%)	4 (5%)	12 (15%)	10 (13%)	27 (35%)	9 (12%)	16 (21%)
Quad	48	10 (21%)	14 (29%)	2 (4%)	8 (17%)	4 (8%)	21 (44%)	4 (8%)	9 (19%)
Penta	30	6 (20%)	12 (40%)	2 (7%)	4 (13%)	6 (20%)	6 (20%)	5 (17%)	7 (23%)
6 Doses/month	51	10 (20%)	15 (29%)	3 (6%)	7 (14%)	5 (10%)	21 (41%)	4 (8%)	11 (2%)
8 Doses/month	27	6 (22%)	11 (41%)	1 (4%)	5 (19%)	5 (19%)	6 (22%)	5 (19%)	5 (19%)

ORR=Objective Response Rate (VGPR+PR), CBR=Clinical Benefit Rate (VGPR+PR+MR), VGPR=Very Good Partial Response, PR=Partial Response, MR=Minor Response, SD=Stable Disease, PD=Progressive Disease, NE=Non-Evaluable
1One patient not included, did not have active myeloma

All responses were adjudicated by an Independent Review Committee (IRC). Among the 78 evaluable patients (median seven prior treatment regimens), the overall response rate (ORR) was 21%, and included very good partial responses (VGPR) and partial responses (PR). Among the 48 patients in the quad-refractory group, the ORR was 21%. For comparison, in a similar quad-refractory patient population, the anti-CD38 monoclonal antibodies Darzalex® and isatuximab had ORRs of 21% and 20%, respectively. Among the 30 patients in the penta-refractory group, the ORR was 20%. Clinical benefit rate (ORR + MR) was 32% (all patients), 29% (quad-refractory), and 37% (penta-refractory). To the Company's knowledge, no other agents have reported response rates in patients with penta-refractory MM. Median overall survival (OS) was 9.3 months for all patients, greater than 11 months (median not reached) for patients with ≥MR, and 5.7 months for patients who did not have any response (≤SD). Median duration of response (DOR) was 5 months. Grade ≥3 cytopenias were the most common side effects and were generally not associated with clinical sequellae. Nausea, anorexia and fatigue were the most common non-hematological side effects, primarily Grades 1 and 2, and were treatable with supportive care and/or dose modification. There were low rates of Grade ≥3 non-hematologic toxicities, with no new safety signals identified. In particular, there was one reported case of Grade 4 infection (1.3%), one case of Grade 2 neuropathy (1.3%) and one reported case of sepsis (1.3%).

Dr. Vogl commented, "The quad- and penta-refractory populations are continuing to expand as patients live longer and cycle through a variety of treatment options, including immunomodulatory drugs, proteasome inhibitors, or anti-CD38 monoclonal antibodies, before their disease ultimately becomes refractory and non-responsive. In my experience, selinexor is the first agent to be specifically investigated in this difficult to treat and currently underserved population. The response rate and duration suggest that selinexor has the potential to be an exciting new option for myeloma treatment."

Karyopharm to Host Multiple Myeloma-focused Dinner Reception and Webcast at ASH 2016

On Monday, December 5, 2016, Karyopharm will host an investor and analyst dinner reception, which will feature a moderated panel discussion with recognized experts in the treatment of MM, updated selinexor data in MM, and a live Q&A session. Confirmed external speakers include:

- Daniel Auclair, PhD (Moderator), *Multiple Myeloma Research Foundation*
- Nizar Bahlis, MD, *University of Calgary, Southern Alberta Cancer Research Institute*
- Paul G. Richardson, MD, *Dana Faber Cancer Institute, Jerome Lipper Multiple Myeloma Center*
- Ravi Vij, MD, MBA, *Washington University School of Medicine in St. Louis, Oncology Division*
- Dan T. Vogl, MD, *Abramson Cancer Center Clinical Research Unit, University of Pennsylvania*

In addition, Michael Kauffman, MD, PhD, CEO of Karyopharm Therapeutics will be joining.

The event will take place during the ASH 2016 annual meeting and interested parties can access a live webcast of the event beginning Monday, December 5, 2016 at 8:15 p.m. PT under "Events & Presentations" in the "Investors" section of the company's website at http://investors.karyopharm.com/events.cfm. A replay of the webcast will be archived on the company's website for 90 days following the event.

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. This reinitiates and amplifies their tumor suppressor function and is believed to lead to the selective induction of apoptosis in cancer cells, while largely sparing normal cells. To date, over 1,800 patients have been treated with selinexor and it is currently being evaluated in several mid- and later-phase clinical trials across multiple cancer indications, including in multiple myeloma in combination with low-dose dexamethasone (STORM) and backbone therapies (STOMP), and in acute myeloid leukemia (SOPRA), diffuse large B-cell lymphoma (SADAL), and liposarcoma (SEAL), among others. Karyopharm plans to initiate a pivotal randomized Phase 3 study of selinexor in combination with bortezomib (Velcade®) and low-dose dexamethasone (BOSTON) in patients with multiple myeloma in early 2017. Additional Phase 1, Phase 2 and Phase 3 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 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, 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), 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, 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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