

Karyopharm Therapeutics Reports Second Quarter Financial Results for 2014

NEWTON, Mass., Aug. 7, 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 reported financial results for the second quarter ended June 30, 2014, and also commented on certain corporate accomplishments and clinical developments plans.

"2014 is a critical year for Karyopharm, as we move our first-in-class, novel, selective inhibitors of nuclear export compounds, or SINE compounds, into additional clinical trials, including registration-directed studies," said Dr. Michael Kauffman, CEO of Karyopharm. "We continue to execute on our clinical and regulatory strategy. The broad and durable anti-tumor activity of our novel XPO1 inhibitors is becoming clearer, and our regulatory pathways towards potential approval are moving forward."

Conference Call Information

Karyopharm will host a conference call today at 4:30 p.m. EDT to discuss the company's second quarter 2014 financial results and provide an update on business activities and development programs. The conference call can be accessed by dialing (855) 437-4406 (domestic) or (484) 756-4292 (international) and by referencing conference ID 82708241. A replay of the conference call will be accessible under "Events & Presentations" in the Investors section of the company's website at www.karyopharm.com.

Recent Corporate Accomplishments

- **Initiated First Registration-Directed Trial.** Karyopharm announced the initiation of its randomized Phase 2 study of Selinexor (KPT-330) in patients 60 years of age or older with relapsed or refractory acute myeloid leukemia (AML) who are ineligible for intensive chemotherapy and/or transplantation. This Selinexor in Older Patients with Relapsed/Refractory AML (SOPRA) study is a randomized trial of Selinexor versus physician's choice, and will be conducted at approximately 50 sites worldwide including sites in the United States, Canada, Europe and Israel. In SOPRA, 150 patients with AML which has relapsed after, or was refractory to, first line therapy will be randomized in a 2:1 fashion to Selinexor provided orally twice per week at a dose of 55mg/m² versus one of four physician choices. Physician choices include best supportive care (BSC) alone, or BSC plus either azacytidine (Vidaza), decitabine (Dacogen), or low dose cytarabine (Ara-C). Overall survival is the primary endpoint. SOPRA is expected to take approximately two years to complete.
- **Phase 2 Combination Study with Ara-C in AML.** This investigator sponsored trial (IST) is evaluating the combination of Selinexor with low dose Ara-C, in newly diagnosed elderly patients with AML or high-risk myelodysplastic syndrome (MDS) who are not eligible for intensive chemotherapy. This combination arm will be compared with a separate arm of low dose Ara-C alone. This IST is being led by Dr. Alan Burnett in the United Kingdom (Cardiff University).
- **Initiated Additional Clinical Trials.** Karyopharm announced the initiation of Phase 1 and Phase 2 studies of Selinexor, including:
 - Phase 1 trial of Selinexor in combination with decitabine (Dacogen) in patients with relapsed or refractory AML and in patients 60 years of age or older with newly diagnosed AML. This IST is being conducted at The Ohio State University Comprehensive Cancer Center under the direction of principal investigator Ramiro Garzon, MD, Associate Professor.
 - *Phase 1 trial of Selinexor in pediatric patients* This first pediatric study of Selinexor is enrolling children with relapsed or refractory acute lymphoblastic leukemia (ALL) or AML. This IST is being led by Dana-Farber/Boston Children's Cancer and Blood Disorders Center under the direction of principal investigator Andrew Place, MD, PhD, Associate Director of Developmental Therapeutics, and has been partially funded by a grant from the William Lawrence & Blanche Hughes Foundation.
 - *Phase 2 trial of Selinexor in patients with advanced gynecologic malignancies including cervical, ovarian and uterine carcinomas* The study, referred to as the SIGN Study (Selinexor In Gynecologic Neoplasms), is being led by Ignace Vergote, MD, at the University Hospitals, Leuven, Belgium. The primary goal of the study is to determine the disease control rate assessed according to RECIST criteria. The secondary goal of the study is to evaluate safety and tolerability. Quality of life will also be evaluated.
 - *Phase 2 trial of Selinexor 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, Andrew Lassman at Columbia University in New York and Patrick Wen at Dana Farber Cancer Institute in Boston. 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.
 - *Phase 2 trial of Selinexor in patients with metastatic hormone-refractory prostate cancer (HRPC)* The Selinexor in Hormone Refractory Indications in Prostate Cancer (SHIP) study is led by Drs. Christopher J. Logothetis and John Araujo of the M.D. Anderson Cancer Center at the University of Texas in Houston and is being funded in part by a grant from the Prostate Cancer Foundation. The primary goal of the study is to determine the disease control rate assessed according to RECIST criteria and the prevention of new bone lesions. The secondary goal of the study is to evaluate the prostate-specific antigen (PSA) response relative to baseline.
- **Follow On Offering.** Karyopharm announced the pricing of its underwritten public offering of 3,044,334 shares of its common stock at a price of \$42.50 per share before underwriting discounts, of which Karyopharm sold 2,844,334 shares of its common stock, including the underwriters' option exercise, and certain existing stockholders sold 200,000 shares of common stock. The base offering and option exercise both closed on July 2, 2014 and Karyopharm received net proceeds of approximately \$112.9 million.
- **Orphan Drug Designation.** During the second quarter, Karyopharm announced that Selinexor received two orphan drug designations from the U.S. Food and Drug Administration (FDA) for the treatment of AML and DLBCL. The designation is designed to encourage the development of drugs to benefit patients suffering from rare diseases. Today, Karyopharm announced it has also received orphan drug designation for AML and DLBCL from the European Medicines Agency (EMA). "The granting of Orphan Drug Designation for AML and DLBCL by both the FDA and EMA is another significant milestone in the Selinexor development program," commented Dr. Sharon Shacham, Founder, CSO and President of Karyopharm.
- **FDA Considers the Effectiveness and Technical Sections Complete to Support Conditional Approval of Verdinexor (KPT-335) in Canine Lymphoma.** Karyopharm announced that the FDA's Center for Veterinary Medicine (CVM) found the effectiveness and safety technical sections complete to support conditional approval under a New Animal Drug Application (NADA) for Karyopharm's oral SINE compound Verdinexor (KPT-335) for the treatment of canine lymphoma in companion dogs. The use of Verdinexor to treat canine lymphoma has been designated a "minor use" in accordance with the Minor Use Minor Species (MUMS) Act. This makes the product eligible for conditional approval similar to orphan drug/accelerated approvals used for submissions of human therapeutics.

- Scientific Updates at Major Conferences. Karyopharm made five clinical presentations at the 50th Annual Meeting of the American Society of Clinical Oncology (ASCO), which included the first presentation of data from its Phase 1b food effect study of Selinexor in patients with advanced sarcomas. In addition, Karyopharm made six presentations at the 19th Congress of the European Hematology Association (EHA), which included the first presentation of clinical data from a combination study of Selinexor, in this case a study of Selinexor in combination with dexamethasone in patients with multiple myeloma.

Clinical Development Plans

In July 2014, Karyopharm met with FDA to discuss the development of Selinexor for the treatment of DLBCL and the planned registration-directed study in DLBCL patients. Based on FDA feedback and data suggesting that some patients may derive long-term benefit from doses well below the highest recommended Phase 2 dose, which is 60mg/m², we have modified our DLBCL registration-directed study design. In this registration-directed Phase 2b Selinexor and Dexamethasone in Aggressive Lymphoma (SADAL) study, approximately 200 patients with DLBCL after 2 to 4 prior lines of immunochemotherapy will be randomized in a 1:1 fashion to low versus high doses of Selinexor given twice weekly. Overall response rate is the primary endpoint, and at least 50% of patients on each arm will have DLBCL of the Germinal-Center B Cell (GCB) subtype, which is less responsive to certain anti-lymphoma therapies.

The dosing regimen for this study has also been simplified. Low dose Selinexor will be a "flat" dose of 60mg (corresponding to approximately 35mg/m²) and high dose Selinexor will be a flat dose of 100mg (corresponding to approximately 60mg/m²). All patients will also receive 8-12mg of dexamethasone as supportive care with each dose of Selinexor in order to reduce anorexia, fatigue and nausea due to Selinexor. Karyopharm believes that this two-arm study design could potentially support an accelerated approval in patients with heavily pretreated, relapsed/refractory DLBCL. The study is expected to begin in the fourth quarter of 2014.

Karyopharm also announced that it has moved Selinexor into earlier lines of DLBCL therapy where Selinexor regimens could be compared with current standards. In particular, the company has begun enrollment of patients with heavily pretreated non-Hodgkin lymphomas into a Phase 1 cohort of Selinexor in combination with the anti-CD20 monoclonal antibody Rituximab. Karyopharm anticipates that Selinexor-Rituximab could be an active regimen against aggressive lymphomas and might be used in future randomized trials. Additional combinations with various chemotherapy and chemo-immunotherapy combinations are planned.

Second Quarter 2014 Financial Results

Cash and cash equivalents as of June 30, 2014, totaled \$132.3 million compared with \$156.0 million as of December 31, 2013.

For the quarter ended June 30, 2014, research and development expense was \$13.2 million compared to \$6.1 million for the same period in the previous year. For the quarter ended June 30, 2014, general and administrative expense was \$3.3 million compared to \$943,000 for the same period in the previous year. The increase in research and development expense resulted primarily from the increase in expenses related to the continued clinical development of Selinexor. The increase in general and administrative expense resulted primarily from the costs of being a public company and an increase in stock-based compensation.

Karyopharm reported a net loss of \$16.4 million, or \$0.55 per share, for the quarter ended June 30, 2014, compared to a net loss of \$6.9 million, or \$2.86 per share, for the same period in the previous year. Net loss includes stock-based compensation expense of \$3.9 million and \$242,000 for the quarters ended June 30, 2014 and 2013, respectively.

For the six months ended June 30, 2014, research and development expense was \$24.1 million compared to \$11.0 million for the same period in the previous year. For the six months ended June 30, 2014, general and administrative expense was \$6.2 million compared to \$1.8 million for the same period in the previous year. The increase in research and development expense resulted primarily from the increase in expenses related to the continued clinical development of Selinexor. The increase in general and administrative expense resulted primarily from the costs of being a public company and an increase in stock-based compensation.

Karyopharm reported a net loss of \$30.1 million, or \$1.02 per share, for the six months ended June 30, 2014, compared to a net loss of \$12.5 million, or \$5.39 per share, for the same period in the previous year. Net loss includes stock-based compensation expense of \$6.7 million and \$446,000 for the six months ended June 30, 2014 and 2013, respectively.

Financial Guidance

Based on current operating plans, Karyopharm expects that its existing cash and cash equivalents, including the proceeds from its July 2014 public offering, will fund its research and development programs and operations into the second half of 2017. Karyopharm expects to end 2014 with greater than \$200 million in cash and cash equivalents.

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. As of August 1, 2014, approximately 400 patients have been treated with Selinexor in Phase 1 and Phase 2 clinical 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 Newton, Massachusetts. For more information about Karyopharm, 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 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.

Karyopharm Therapeutics Inc.

Unaudited Selected Consolidated Balance Sheet Information
(in thousands)

	June 30, 2014	December 31, 2013
Cash and cash equivalents	\$132,307	\$155,974
Prepaid expenses and other current assets	2,976	1,982
Property and equipment, net	604	240
Other assets	1,792	30
Total assets	\$137,679	\$158,226
Accounts payable and accrued expenses	\$5,459	\$2,908
Other liabilities	540	384
Stockholders' equity	131,680	154,934
Total liabilities and stockholders' equity	\$137,679	\$158,226

Karyopharm Therapeutics Inc.

Unaudited Condensed Consolidated Statement of Operations
(in thousands, except share and per share data)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2014	2013	2014	2013
Revenue:				
Contract and grant revenue	\$21	\$133	\$193	\$366
Operating expenses:				
Research and development	13,159	6,060	24,138	11,025
General and administrative	3,310	943	6,214	1,822
Total operating expenses	16,469	7,003	30,352	12,847
Loss from operations	(16,448)	(6,870)	(30,159)	(12,481)
Interest income	17	1	34	1
Net loss	(\$16,431)	(\$6,869)	(\$30,125)	(\$12,480)
Net loss per share applicable to common stockholders-basic and diluted	(\$0.55)	(\$2.86)	(\$1.02)	(\$5.39)
Weighted-average number of common shares used in net loss per share applicable to common stockholders-basic and diluted	29,659,457	2,404,080	29,633,215	2,315,331

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