Karyopharm Reports Second Quarter 2017 Financial Results and Highlights Recent Progress

– Pivotal Phase 3 BOSTON Study Underway -
– Updated Phase 1b/2 STOMP Data Expected by Year End 2017; Top-line Phase 2b STORM Data Expected by April 2018 -
– Phase 2 SEAL Hazard Ratio Expected in September/October 2017, Along with Other Pipeline Program Updates During the Second Half of 2017 -
– Conference Call Scheduled for Today at 8:30 a.m. ET –

NEWTON, Mass., Aug. 08, 2017 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a clinical-stage pharmaceutical company, today reported financial results for the second quarter 2017 and commented on recent accomplishments and clinical development plans for its lead, novel, oral Selective Inhibitor of Nuclear Export (SINE™) compound selinexor (KPT-330), and other pipeline assets verdinexor (KPT-335), and KPT-9274, its oral, dual inhibitor of p21-activated kinase 4 (PAK4) and nicotinamide phosphoribosyltransferase (NAMPT).

"Our second quarter achievements marked significant progress across several of our development programs, and especially for selinexor," saidMichael G. Kauffman, MD, PhD, Chief Executive Officer of Karyopharm. "At the 2017European Hematology Association (EHA) Annual Meeting, we reported updated data from the Phase 2b SADAL study investigating selinexor in patients with relapsed or refractory diffuse large B-cell lymphoma (DLBCL). The overall response rate (ORR) increased to 33.3% for the overall trial population with similar response rates in patients with double- or triple-hit DLBCL, indicating clear activity in this population which usually has a particularly poor prognosis. As we move to the second half of the year, our focus remains on execution of key later-stage trials in our lead indications of multiple myeloma (MM), DLBCL and liposarcoma. In myeloma, the pivotal Phase 3 BOSTON study is now underway. The Phase 2b STORM study, for possible accelerated approval, continues to enroll well with top-line data expected by April 2018. In liposarcoma, the Phase 2 portion of the blinded, randomized Phase 2/3 SEAL study recently completed enrollment and we look forward to reporting the hazard ratio for progression-free survival (PFS) and providing an update regarding the planned development path in this indication during September or October 2017."

Second Quarter 2017 and Recent Events, Highlights and Milestones:

Selinexor in Multiple Myeloma

- Pivotal Phase 3 BOSTON Study Initiated. Karyopharm initiated the pivotal, randomized Phase 3 BOSTON (Bortezomib, Selinexor and dexamethasone) study, evaluating once weekly selinexor 100mg in combination with the proteasome inhibitor Velcade (bortezomib, once weekly) and dexamethasone (SVd), compared to standard dose Velcade (twice weekly) and low-dose dexamethasone (Vd) in patients with MM who have had one to three prior lines of therapy. The primary endpoints of the study are PFS and ORR. The BOSTON study is expected to enroll approximately 360 patients at over 100 clinical sites internationally. Karyopharm is projecting to complete enrollment in 2018, with top-line data anticipated in 2019.

- Selinexor Named Among the "Top 5 Oncology R&D Products Worldwide in 2022" by EvaluatePharma®. In EvaluatePharma's recent report,*World Preview 2017, Outlook to 2022*, selinexor was projected to be one of the top five selling oncology research and development products worldwide in 2022, with the potential to generate estimated revenues of $920 million in worldwide annual sales and capture 0.5% of the worldwide oncology market share in the same timeframe. This analysis is based on EvaluatePharma's coverage of the world's 6,500 leading pharmaceutical and biotech companies and highlights certain important industry trends by therapy area.

- Ongoing Phase 2b STORM Study Expansion in Patients with Penta-refractory MM. The Phase 2b STORM study, which was recently expanded to include 122 additional patients with penta-refractory MM, continues to enroll on track. Karyopharm expects to report top-line data from the expanded cohort by April 2018, and, assuming a positive outcome, intends to use the data from the expanded STORM study to support a request for accelerated approval for selinexor in heavily pretreated MM.

- Ongoing Phase 1b/2 STOMP Study Evaluating Selinexor in Combination with Several Key MM Drugs. Enrollment is complete in the Phase 1b/2 STOMP arm evaluating selinexor in combination with Velcade and low-dose dexamethasone (SVd) in heavily pretreated patients with MM. The SVd arm of the STOMP study enrolled 42 patients. Dose escalation is complete and expansion is ongoing in the arms evaluating oral selinexor plus immunomodulatory drug (IMID) combinations, including selinexor + Revlimid® (lenalidomide) + dexamethasone (SRd), and selinexor + Pomalyst® (pomalidomide) and dexamethasone (SPd). The Company expects to report updated data on these convenient, all oral regimens by year end 2017.

- New Study Arm Initiated in Phase 1b/2 STOMP Study Evaluating Selinexor in Combination with Darzalex® (daratumumab). Karyopharm has dosed patients in a new Phase 1b/2 STOMP study arm designed to evaluate selinexor in combination with the anti-CD38 monoclonal antibody Darzalex and low-dose dexamethasone (SDd) in heavily pretreated patients with MM. The SDd arm of the STOMP study is expected to enroll up to 16 patients and the Company expects to report top-line data in the first half of 2018.

Selinexor in Diffuse Large B-Cell Lymphoma

- Updated Data from Phase 2b SADAL Study in DLBCL Presented at EHA 2017. At the 2017 EHA Annual Meeting in June, an oral presentation was given that highlighted updated data from the ongoing Phase 2b SADAL study evaluating single-agent selinexor in patients with relapsed or refractory DLBCL. This latest data demonstrated that selinexor achieved an ORR of 33.3% and a duration of response (DOR) of &#62 7 months in the first 63 patients, as adjudicated by an independent central radiological committee. Patients were randomized to one of two single-agent selinexor arms, a higher dose arm of 100 mg twice weekly and a lower dose arm of 60 mg twice weekly. The median overall survival was 8 months for all patients, consistent with published data in this population which has a very poor prognosis. As of the data cutoff date, the median survival for the responders had not been reached and was over 9 months. Most responses occurred at the first response evaluation (~2 months). As of the data cutoff date, 9 of the 21 responding patients remained on treatment, including 6 patients who had a complete response (CR). Selinexor also showed robust, single-agent activity against GCB and non-GCB subtypes of DLBCL. Of the 32 patients with DLBCL of the GCB-subtype, 9 responded (4 patients with a CR, 5 patients with a partial response (PR)) for an ORR of 28.1%. Of the 31 patients with DLBCL of the non-GCB (or ABC)-subtype, 12 responded (5 patients with a CR, 7 patients with a PR) for an ORR of 38.7%. Amongst the 14 patients with "double-" or "triple-hit" DLBCL, the ORR was consistent with the ORR across the SADAL patient population, indicating anti-cancer activity in this population, which usually has a particularly poor prognosis. Side effects were consistent with those previously reported with selinexor, and no new safety signals were identified. Importantly, side effects were reduced in the 60mg cohort in comparison with the 100mg cohort.

  In consultation with the U.S. Food and Drug Administration (FDA), Karyopharm amended the SADAL study, removing the 100mg arm and continuing enrollment only in the 60mg twice weekly arm. The FDA has agreed that the single-arm trial design appears appropriate for accelerated approval in DLBCL, though eligibility for accelerated approval will depend on the complete trial results and available therapies at the time of regulatory action. The SADAL study is expected to enroll up to a total of 130 patients in the 60mg single-arm cohort and Karyopharm plans to report top-line results in the

second half of 2018.

Selinexor in Other Hematologic Malignancies

Published Phase 1 Data Demonstrating Selinexor's Activity in Patients with Relapsed/Refractory Non-Hodgkin's Lymphoma (NHL) in the Journal Blood. A paper describing results from the first in human Phase 1 clinical study assessing safety and preliminary activity of selinexor in patients with relapsed or refractory NHL was recently published in the journal Blood. In the paper, authored by John Kuruvilla, et al., titled "Selective inhibition of nuclear export with selinexor in patients with non-Hodgkin's lymphoma," Karyopharm collaborators reported that selinexor was generally well tolerated. Of the 70 evaluable patients, 22 (31%) achieved an objective response (OR), including 4 CRs and 18 PRs, which were observed across a spectrum of NHL subtypes, including DLBCL, Richter's transformation, mantle cell lymphoma, follicular lymphoma and chronic lymphocytic leukemia. All four CRs were in patients with DLBCL, and two of the four patients are believed to have remained relapse-free as of the publication date, greater than 3 years since initiation of single agent selinexor therapy. Tumor biopsies showed decreases in cell signaling pathways, reduced proliferation, nuclear localization of XPO1 cargos and increased apoptosis after treatment. The most common grade 3-4 drug-related AEs were thrombocytopenia (47%), neutropenia (32%), anemia (27%), leukopenia (16%), fatigue (11%) and hyponatremia (10%). A maximum tolerated dose was not defined, but the highest allowable dose was ~120 mg twice weekly. Based on both tolerability and antitumor activity, the recommended Phase 2 dose of selinexor in NHL is 35 mg/m2 (~60 mg) twice weekly.

Selinexor in Solid Tumors

- Ongoing Phase 2/3 SEAL Study in Liposarcoma. Enrollment is now complete in the Phase 2 portion of the blinded, randomized Phase 2/3 SEAL study evaluating single-agent selinexor versus placebo in patients with advanced liposarcoma. Karyopharm expects to report the hazard ratio for PFS from the Phase 2 portion of the SEAL study and providing an update regarding the planned development path in this indication during September or October 2017. The primary endpoint of the SEAL study is PFS and both the trial design and endpoints have been accepted by the FDA and the European Medicines Agency.

- Oral Presentation Highlighting Efficacy, Safety and Intratumoral Pharmacokinetic Data for Selinexor in Glioblastoma at the 2017 World Federation of Neuro-Oncology Societies (WFNOS) Meeting. Clinical data from a Phase 2 study evaluating selinexor in patients with recurrent glioblastoma was highlighted in an oral presentation at the 2017 WFNOS meeting by Andrew Lassman, MD, Columbia University Medical Center. The data demonstrated that oral selinexor achieved responses and sufficient intratumoral penetration, with a manageable tolerability profile when accompanied by standard supportive care. Importantly, disease control rates using selinexor dosed at 80 mg once weekly were as high or higher than those observed with more intensive dosing, and tolerability was improved.

Verdinexor

- Signed Global License Agreement with Anivive Lifesciences for Verdinexor for Animal Health Applications. Karyopharm and Anivive, a privately-held biotech company, executed a licensing agreement under which Anivive licensed from Karyopharm exclusive worldwide rights to research, develop and commercialize verdinexor for the treatment of cancer in companion animals. Under the terms of the agreement, Anivive made a one-time upfront payment of $1 million to Karyopharm. Anivive also agreed to pay up to an additional $43.5 million based on technology transfer and achievement of specified regulatory, clinical and commercial milestones, assuming approval in both the U.S. and the European Union. In addition, Anivive agreed to pay Karyopharm a low double-digit royalty based on future net sales of verdinexor.

KPT-9274

- Preclinical Efficacy Highlighting KPT-9274's Anti-Cancer Activity in Dogs with Spontaneous Lymphomas Presented as a Late-Breaking Poster at the American Association of Cancer Research (AACR) 2017 Annual Meeting. At the AACR 2017 Annual Meeting in April, Karyopharm collaborator Cheryl London of Tufts University presented a late-breaking poster highlighting preclinical data demonstrating the activity and synergy of KPT-9274, the Company's oral dual inhibitor of PAK4/NAMPT, with doxorubicin to treat dogs with lymphoma. KPT-9274 is currently being evaluated in a Phase 1 safety and tolerability study in patients with advanced solid malignancies (including sarcoma, colon and lung cancer) or non-Hodgkin's lymphoma (NHL) whose disease has relapsed after standard therapy(s). Top-line data from this clinical study are expected later this year.

Other Corporate and Clinical Developments

- Generated $52.3 Million in Equity Financings. In April 2017, the Company sold approximately 3.9 million shares of common stock in an underwritten public offering at a price to the public of $10.25 per share, resulting in net proceeds to the Company of approximately $37.9 million after deducting underwriting discounts and commissions and other offering expenses, and sold approximately 1.3 million shares under its ATM offering facility for net proceeds of approximately $14.4 million.

Second Quarter 2017 Financial Results

Cash, cash equivalents and investments as of June 30, 2017, including restricted cash, totaled $181.2 million, compared to $175.5 million as of December 31, 2016.

On April 28, 2017, Karyopharm completed an underwritten public offering of 3,902,439 shares of its common stock at a price to the public of $10.25 per share. The net proceeds to Karyopharm from the offering, after deducting the underwriting discounts and commissions and offering expenses, were approximately $37.9 million. In addition, during April 2017, the Company sold approximately 1.3 million shares under its ATM offering facility for net proceeds of approximately $14.4 million.

For the quarter ended June 30, 2017, research and development expense was $23.1 million compared to $24.6 million for the quarter ended June 30, 2016. For the quarter ended June 30, 2017, general and administrative expense was $6.6 million compared to $6.0 million for the quarter ended June 30, 2016.

Karyopharm reported a net loss of $29.4 million, or $0.64 per share, for the quarter ended June 30, 2017, compared to a net loss of $30.2 million, or $0.84 per share, for the quarter ended June 30, 2016. Net loss includes stock-based compensation expense of $5.1 million and $6.4 million for the quarters ended June 30, 2017 and June 30, 2016, respectively.

Financial Outlook

Karyopharm expects its operating cash burn, including research and development and general and administrative expenses, for the year ending December 31, 2017 to be in the range of $90-95 million. Based on current operating plans, Karyopharm expects that its existing cash and cash equivalents will be sufficient to fund its research and development programs and operations into 2019, including the continued clinical development of selinexor in the Company's lead indications with a focus on filing for accelerated approvals for both MM and DLBCL during 2018, and preparing a commercial infrastructure for the potential launch of selinexor in North America and Western Europe.

Conference Call Information:

Karyopharm will host a conference call today, Tuesday, August 8, 2017, at 8:30 a.m. Eastern Time, to discuss the second quarter 2017 financial results, recent accomplishments, clinical developments and business plans. To access the conference call, please dial (855) 437-4406 (US) or (484) 756-4292 (international) at least five minutes prior to the start time and refer to conference ID: 53128722. An audio recording of the call will be available under "Events & Presentations" in the "Investor" section of Karyopharm's website, http://www.karyopharm.com, approximately two hours after the event.

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). The Company's initial focus is on seeking regulatory approval and commercialization of its lead drug candidate, oral selinexor (KPT-330). To date, over 2,100 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 a pivotal, randomized Phase 3 study in combination with Velcade® (bortezomib) and low-dose dexamethasone (BOSTON), in combination with low-dose dexamethasone (STORM) and backbone therapies (STOMP), and in diffuse large B-cell lymphoma (SADAL) and liposarcoma (SEAL), among others. 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. 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 five 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 and enrollment of certain trials and of the reporting of data from such trials, and Karyopharm's financial outlook and financial projections for Karyopharm. Such statements are subject to numerous important factors, risks and uncertainties that may cause actual events or results to differ materially from Karyopharm's current expectations. For example, there can be no guarantee that any of Karyopharm's SINE™ compounds, including selinexor (KPT-330) or KPT-9274, Karyopharm's first-in-class oral dual inhibitor of PAK4 and NAMPT, 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 FDA 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 March 31, 2017, which was filed with the Securities and Exchange Commission (SEC) on May 4, 2017, 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, except as required by law, Karyopharm expressly disclaims any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise.

CONDENSED CONSOLIDATED BALANCE SHEETS
(unaudited)
(in thousands, except share and per share amounts)

	June 30, 2017	December 31, 2016
Assets		
Current assets:		
Cash and cash equivalents	$ 55,381	$ 49,663
Short-term investments	88,073	79,889
Restricted cash	200	—
Prepaid expenses and other current assets	2,070	2,084
Total current assets	145,724	131,636
Property and equipment, net	2,473	2,836
Long-term investments	37,269	45,434
Restricted cash	284	479
Total assets	$ 185,750	$ 180,385
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 3,247	$ 4,751
Accrued expenses	12,876	11,362
Deferred revenue	1,025	—
Deferred rent	292	280
Other current liabilities	80	83
Total current liabilities	17,520	16,476
Deferred rent, net of current portion	1,516	1,666

Total liabilities	19,036	18,142
Stockholders' equity:		
Preferred stock, $0.0001 par value; 5,000,000 shares authorized; none issued and outstanding	—	—
Common stock, $0.0001 par value; 100,000,000 shares authorized; 47,123,208 and 41,887,829 shares issued and outstanding at June 30, 2017 and December 31, 2016, respectively	5	4
Additional paid-in capital	592,534	528,617
Accumulated other comprehensive loss	(165)	(274)
Accumulated deficit	(425,660)	(366,104)
Total stockholders' equity	166,714	162,243
Total liabilities and stockholders' equity	$ 185,750	$ 180,385

Karyopharm Therapeutics Inc.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
(unaudited)
(in thousands, except share and per share amounts)

	Three Months Ended, June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Contract and grant revenue	$ 3	$ 59	$ 71	$ 59
Operating expenses:				
Research and development	23,120	24,579	47,203	46,374
General and administrative	6,635	5,956	12,899	11,510
Total operating expenses	29,755	30,535	60,102	57,884
Loss from operations	(29,752)	(30,476)	(60,031)	(57,825)
Other income (expense):				
Interest income	412	329	812	615
Other expense	(29)	(11)	(44)	(7)
Total other income, net	383	318	768	608
Loss before income taxes	(29,369)	(30,158)	(59,263)	(57,217)
Provision for income taxes	(18)	—	(41)	—
Net loss	$ (29,387)	$ (30,158)	$ (59,304)	$ (57,217)
Net loss per share—basic and diluted	$ (0.64)	$ (0.84)	$ (1.35)	$ (1.59)
Weighted-average number of common shares outstanding used in net loss per share—basic and diluted	45,831,239	35,956,470	43,873,892	35,917,486

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