November 2, 2017

Karyopharm Reports Third Quarter 2017 Financial Results and Highlights Recent Progress

− Company Completes Exclusive Licensing Transaction Valued at Up To $193 Million Plus Royalties with Ono Pharmaceutical Co. Ltd. for Selinexor and KPT-8602 In Japan and Certain Other Countries in Asia —

− Michael Falvey Named Chief Financial Officer—

− Fourteen Abstracts Selected for ASH 2017, Including Three Oral Presentations —

− Conference Call Scheduled for Today at 8:30 a.m. ET −

NEWTON, Mass., Nov. 02, 2017 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a clinical-stage pharmaceutical company, today reported financial results for the third quarter 2017 and provided an overview of recent accomplishments and clinical development plans for its lead, novel, oral SINE™ compound selinexor (KPT-330), and other pipeline assets including KPT-8602, its second-generation oral SINE™ compound, KPT-9274, its oral, dual inhibitor of p21-activated kinase 4 (PAK4) and nicotinamide phosphoribosyltransferase (NAMPT).

"We are very proud of our accomplishments to date in 2017, especially the recent execution of an exclusive licensing transaction, valued at up to $193 million, plus royalties, with Ono Pharmaceutical Co. Ltd. for the development and commercialization of selinexor and KPT-8602 for all human oncology indications in Japan and certain other countries in Asia," said Michael G. Kauffman, MD, PhD, Chief Executive Officer of Karyopharm. "As we head into year end, we look forward to reporting clinical data from multiple treatment arms of the Phase 1b/2 STOMP study evaluating oral selinexor in combination with several current 'backbone' therapies for multiple myeloma (MM) at this year's American Society of Hematology (ASH) Annual Meeting. Fourteen abstracts highlighting data for selinexor and our other pipeline assets have been selected for presentation at ASH, and we are pleased to be able to share these data with the medical community at the meeting this year. We continue to execute on the Phase 2b STORM study on schedule, and we expect to report top-line results by April 2018."

Third Quarter 2017 and Recent Events, Highlights and Milestones:

Partnerships and Other Key Corporate Developments

- **Signed Exclusive License Agreement with Ono Pharmaceutical Co., Ltd (Ono) to Develop and Commercialize Selinexor and KPT-8602 in Japan and Other Countries in Asia.** In October 2017, Karyopharm announced its entry into an exclusive license agreement with Ono for the development and commercialization of selinexor and KPT-8602, the Company's second-generation oral SINE™ compound. The agreement includes the development of selinexor and KPT-8602 for the diagnosis, treatment and/or prevention of all human oncology indications in Japan, South Korea, Taiwan, Hong Kong, and the ASEAN countries (the Territory). The transaction, which carries a total deal value of up to $193.0 million based on the exchange rate on the effective date of the license agreement, includes a one-time upfront payment of ¥2.5 billion ($22.3 million) from Ono to Karyopharm and up to an additional ¥19.15 billion ($170.7 million) if certain specified future development and commercial milestones are achieved by Ono. In addition, Karyopharm is also eligible to receive low double-digit royalties based on future net sales of selinexor and KPT-8602 in the Territory. In exchange, Ono received exclusive rights to develop and commercialize both compounds in the Territory, at its own cost and expense. Ono will also have the ability to participate in any global clinical study of selinexor and KPT-8602, and will bear the cost and expense for patients enrolled in clinical studies in the Territory. Karyopharm retains all rights to selinexor and KPT-8602 outside the Territory.

- **Michael Falvey Appointed Chief Financial Officer.** In September 2017, Karyopharm announced the appointment of Michael Falvey as Executive Vice President, Chief Financial Officer and Treasurer. Mr. Falvey brings 35 years of experience in executing business growth and financial strategies for publicly-traded and privately-held companies, including senior financial leadership roles at healthcare-focused, scientific organizations. Mr. Falvey leads the Company's financial and capital markets strategy, as well as advises on business development and transactional activities.

- **Other Key Personnel Appointments.** In September 2017, Karyopharm also announced the appointment of Jatin Shah, MD, as Vice President, Clinical Strategy and Joan Wood as Chief Human Resources Officer. Dr. Shah brings significant medical oncology experience, including treating patients with MM and clinical research. Dr. Shah formerly served as Associate Professor and Director of the Myeloma Clinical/Translational Research Department at MD Anderson Cancer Center. Ms. Wood is an experienced human resources executive with significant experience in global talent management in the biopharma industry. Prior to joining Karyopharm, she served in senior leadership roles at Sarepta Therapeutics and Genzyme Corporation.

Selinexor in Multiple Myeloma

- **Phase 1b/2 STOMP Data Selected for Presentation at ASH 2017.** Four abstracts featuring clinical data from the four treatment arms of the ongoing Phase 1b/2 STOMP study have been selected for poster presentations at the upcoming ASH 2017 annual meeting in early December (ASH 2017). The poster presentations will include updated data from the arm evaluating selinexor in combination with Velcade® (bortezomib) and low-dose dexamethasone (SVd); updated data from the arm evaluating selinexor in combination with Pomalyst® (pomalidomide) and low-dose dexamethasone (SPd); updated data from the arm evaluating selinexor in combination with Revlimid® (lenalidomide) and low-dose dexamethasone (SRd); and preliminary data from the arm evaluating selinexor in combination with Darzalex® (daratumumab) and low-dose dexamethasone (SDd).

- **Several Investigator-sponsored Trial and Preclinical Abstracts Selected for Presentation at ASH 2017.** Four abstracts featuring clinical data from investigator-sponsored clinical studies evaluating selinexor either as a single-agent or in combination with other anti-cancer agents for the treatment of acute myeloid leukemia (AML), myelodysplastic syndrome (MDS) and MM have been selected for oral or poster presentations at ASH 2017. In addition, three abstracts describing preclinical research exploring the use of selinexor in models of AML, lymphoma, mantle cell lymphoma (MCL) and MM have also been selected for oral or poster presentations at the meeting.

- **Pivotal Phase 3 BOSTON Study Underway.** Karyopharm's pivotal, randomized Phase 3 BOSTON (**B**ortezomib, **S**elinexor and dexame**t**has**on**e) study is now underway. BOSTON is designed to evaluate 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 progression-free survival (PFS) and overall response rate (ORR). Both the trial design and endpoints have been agreed to by the U.S. Food and Drug Administration (FDA) and the European Medicines Agency as acceptable for approval. The Company expects to enroll approximately 360 patients at over 100 clinical sites internationally and is projecting completion of enrollment in 2018, with top-line data anticipated in 2019.

- **Ongoing Phase 2b STORM Study Expansion in Patients with Penta-refractory MM.** The Phase 2b STORM study, which was previously 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 oral selinexor in this heavily pretreated MM patient population.

Selinexor in Diffuse Large B-Cell Lymphoma

- **Ongoing Phase 2b SADAL Study in DLBCL.** Karyopharm is also investigating oral selinexor as a single-agent for the treatment of patients with relapsed or refractory diffuse large B-cell lymphoma (DLBCL). The Company recently reported positive updated Phase 2b SADAL study results (n=63) where selinexor demonstrated an ORR of 33% for the overall study population and 35% in patients with "double-" or "triple-hit" DLBCL, indicating anti-cancer activity in this patient population who usually have a particularly poor prognosis. Median duration of response (DOR) for the overall study population was greater than 7 months. Side effects were consistent with those previously reported with selinexor, with no new safety signals identified. The SADAL study is expected to enroll up to a total of 130 patients in the single-arm cohort evaluating single-agent selinexor dosed 60mg twice weekly in patients with two or more lines of prior therapy. Karyopharm plans to report top-line results in the second half of 2018 and, assuming a positive outcome, the Company intends to use the data from the SADAL study to support a request for accelerated approval for oral selinexor in this relapsed/refractory DLBCL patient population.

Selinexor in Solid Tumors

- **Phase 3 Portion of the Phase 2/3 SEAL Study in Liposarcoma Underway Following Successful Outcome from Phase 2 Portion.** Karyopharm recently reported a successful outcome from the Phase 2 portion of the blinded, randomized Phase 2/3 SEAL study evaluating single-agent selinexor versus placebo in patients with previously treated, advanced unresectable dedifferentiated liposarcoma. For the SEAL study's primary endpoint of PFS, oral selinexor showed superiority over placebo, achieving a hazard ratio (HR) of 0.60, representing a 40%

reduction in the risk of progression or death, as assessed by Independent Central Radiological Review (ICRR) (RECIST v1.1). Oral selinexor demonstrated an expected and manageable safety profile with no new or unexpected safety signals identified. The majority of treatment-related adverse events were low grade and reversible with dose modifications and/or standard supportive care. Importantly, the incidence of infections in the selinexor arm was less than that reported in the placebo arm.

The Phase 3 portion of the SEAL study, which was originally initiated in North America, is ongoing and has been expanded to include Europe. In this blinded, placebo-controlled Phase 3 study, up to 222 patients are expected to be enrolled and randomized 2:1 to receive either oral selinexor, (60mg twice weekly) until disease progression or intolerability, or placebo. Patients whose disease progresses on placebo will be permitted to cross over to the selinexor arm. The primary endpoint of the Phase 3 portion of the study is PFS (RECIST v1.1) as assessed by the ICRR. The Phase 3 study design and primary endpoint of PFS were agreed to by the FDA. Top-line data from the Phase 3 portion of the SEAL study are anticipated by the end of 2019. Assuming a positive outcome, these data are expected to support a New Drug Application for oral selinexor as a potential new treatment for patients with advanced unresectable dedifferentiated liposarcoma.

- **Poster Presentation Featuring Selinexor Phase 1 Safety and Tolerability Data in Ovarian and Endometrial Cancers at the European Society of Medical Oncology 2017 Annual Meeting (ESMO 2017).** Top-line Phase 1 data from this ongoing investigator-sponsored study evaluating selinexor in combination with paclitaxel and carboplatin, was presented by Dr. Vikky Makker, Memorial Sloan Kettering Cancer Center, and showed encouraging early efficacy and a manageable safety profile in patients with advanced ovarian and endometrial cancers. A recommended Phase 2 dose (RP2D) regimen for selinexor was established (60mg once weekly in combination with carboplatin AUC5 on Day 1 and paclitaxel 80 mg/m2 on Days 1, 8 and 15 of each 21-day cycle) and expansion cohorts for the RP2D regimen are planned.

KPT-8602

- **Two Abstracts Highlighting KPT-8602 Preclinical Research Selected for Presentation at ASH 2017.** Two abstracts describing preclinical research exploring the use of KPT-8602 in models of MM and myelofibrosis have been selected for poster presentations at the meeting.

KPT-9274

- **Preclinical Research for KPT-9274 Selected for Oral Presentation at ASH 2017.** An abstract describing preclinical research exploring the use of KPT-9274 in a model of Waldenstrom macroglobulinemia has been selected for an oral presentation at the meeting.

- **Poster Presentation Featuring KPT-9274 Phase 1 Safety and Tolerability Data in Advanced Solid Malignancies or Relapsed NHL at ESMO 2017.** Top-line Phase 1 data from this ongoing study evaluating KPT-9274 in patients with advanced solid malignancies (including sarcoma, colon and lung cancer) or relapsed non-Hodgkin's lymphoma (NHL) following standard therapy(s), was presented by Dr. Aung Naing, MD Anderson Cancer Center, and showed a manageable safety profile and early signals of anti-tumor activity. The poster also reported findings that niacin can be safely administered with KPT-9274 and may improve tolerability, particularly with respect to anemia. These study findings indicate that in patients whose disease has progressed despite most available therapies, KPT-9274 can induce tumor shrinkage and disease stabilization. Dose escalation remains ongoing.

Third Quarter 2017 Financial Results

Cash, cash equivalents and investments as of September 30, 2017, including restricted cash, totaled $159.4 million, compared to $175.5 million as of December 31, 2016.

For the quarter ended September 30, 2017, research and development expense was $25.2 million compared to $19.9 million for the quarter ended September 30, 2016. For the quarter ended September 30, 2017, general and administrative expense was $5.8 million compared to $5.9 million for the quarter ended September 30, 2016.

Karyopharm reported a net loss of $30.6 million, or $0.65 per share, for the quarter ended September 30, 2017, compared to a net loss of $25.4 million, or $0.69 per share, for the quarter ended September 30, 2016. Net loss includes stock-based compensation expense of $4.9 million and $5.6 million for the quarters ended September 30, 2017 and September 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 approximately $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 a new drug application with the FDA requesting accelerated approval in MM during 2018, assuming positive data from the STORM study, and preparing the commercial infrastructure for the potential launch of selinexor in North America and Western Europe. Additional key activities for 2018 include topline data from the SADAL study targeted for the second half of 2018 and continued enrollment in the Phase 3 BOSTON and SEAL studies.

Conference Call Information

Karyopharm will host a conference call today, Thursday, November 2, 2017, at 8:30 a.m. Eastern Time, to discuss the third quarter 2017 financial results, recent accomplishments, clinical developments and business plans. To access the conference call, please dial (855) 437-4406 or (484) 756-4292 (international) at least five minutes prior to the start time and refer to conference ID: 98527624. 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 2,200 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 potential to receive milestone and royalty payments under the license agreement with Ono; the success of Karyopharm's arrangement with Ono and the parties' ability to work effectively together; therapeutic potential of and potential clinical development plans for Karyopharm's drug candidates, including the timing of 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), KPT-8602, Karyopharm's second-generation oral SINE™ compound, 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; the ability of Karyopharm or Ono to fully perform their respective obligations under the license agreement; the impact of volatility in currency exchange rates; 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 June 30, 2017, which was filed with the Securities and Exchange Commission (SEC) on August 8, 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.

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Revlimid® and Pomalyst® are registered trademarks of Celgene Corporation
Darzalex® is a registered trademark of Janssen Biotech, Inc.

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Karyopharm Therapeutics Inc.
CONDENSED CONSOLIDATED BALANCE SHEETS
(unaudited)
(in thousands, except share and per share amounts)

	September 30, 2017	December 31, 2016
Assets		
Current assets:		
Cash and cash equivalents	$ 54,450	$ 49,663
Short-term investments	64,956	79,889
Restricted cash	200	—
Prepaid expenses and other current assets	2,076	2,084
Total current assets	121,682	131,636
Property and equipment, net	2,304	2,836
Long-term investments	39,498	45,434
Restricted cash	289	479
Total assets	$ 163,773	$ 180,385
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 1,890	$ 4,751
Accrued expenses	17,715	11,362
Deferred revenue	1,050	—
Deferred rent	298	280
Other current liabilities	202	83
Total current liabilities	21,155	16,476
Deferred rent, net of current portion	1,441	1,666
Total liabilities	22,596	18,142
Stockholders' equity:		
Preferred stock, $0.0001par value; 5,000,000 shares authorized; none issued and outstanding	—	—
Common stock, $0.0001 par value; 100,000,000shares authorized; 47,154,204 and 41,887,829 shares issued and outstanding at September 30, 2017 and December 31, 2016, respectively	5	4
Additional paid-in capital	597,562	528,617
Accumulated other comprehensive loss	(90)	(274)

Accumulated deficit	(456,300)	(366,104)
Total stockholders' equity	141,177	162,243
Total liabilities and stockholders' equity	$ 163,773	$ 180,385

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2017	2016	2017	2016
Contract and grant revenue	$ —	$ 48	$ 71	$ 107
Operating expenses:				
Research and development	25,237	19,893	72,440	66,267
General and administrative	5,818	5,897	18,717	17,407
Total operating expenses	31,055	25,790	91,157	83,674
Loss from operations	(31,055)	(25,742)	(91,086)	(83,567)
Other income (expense):				
Interest income	454	311	1,266	926
Other income (expense)	(26)	6	(70)	(1)
Total other income (expense), net	428	317	1,196	925
Loss before income taxes	(30,627)	(25,425)	(89,890)	(82,642)
Provision for income taxes	(13)	—	(54)	—
Net loss	$ (30,640)	$ (25,425)	$ (89,944)	$ (82,642)
Net loss per share—basic and diluted	$ (0.65)	$ (0.69)	$ (2.00)	$ (2.28)
Weighted-average number of common shares outstanding used in net loss per share—basic and diluted	47,141,146	36,819,329	44,974,945	36,223,324