Karyopharm Reports Fourth Quarter and Full Year 2017 Financial Results and Highlights Recent Progress

− Phase 2b STORM Study Evaluating Selinexor in Patients with Penta-Refractory Myeloma Remains on Track; Top-Line Data Expected end of April 2018 –

− Company Executes Two High-Value Strategic Transactions Providing Substantial Validation for Its Lead Assets and XPO1 Inhibition –

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

NEWTON, Mass., March 15, 2018 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a clinical-stage pharmaceutical company, today reported financial results for the fourth quarter and full year 2017 and provided an overview of recent accomplishments and clinical development plans for selinexor, its lead, novel, oral SINE compound, and eltanexor, its second-generation oral SINE compound.

“2017 was a year of significant achievement for Karyopharm where we reported positive clinical data from our lead selinexor programs, including the Phase 1b/2 STOMP study in multiple myeloma (MM), the Phase 2b SADAL study in diffuse large B-cell lymphoma (DLBCL), and the Phase 2/3 SEAL study in liposarcoma, and the subsequent advancement of each study,” said Michael G. Kauffman, MD, PhD, Chief Executive Officer of Karyopharm. “We are looking forward to an event-driven 2018 beginning at the end of April with top-line results from the Phase 2b STORM study, followed by top-line data from the Phase 2b SADAL study by the year end. Assuming positive outcomes, we plan to use the data from both of these studies to support requests for accelerated approval from the U.S. Food and Drug Administration (FDA) and conditional approval from the European Medicines Agency (EMA) in these indications. On the corporate front, we continue to build out our commercial team in anticipation of a potential selinexor launch in the U.S. in 2019.”

Fourth Quarter 2017 and Recent Events

New Strategic Relationships

- **Biogen’s Acquisition of KPT-350 for the Treatment of Neurological and Neurodegenerative Diseases.** In January 2018, Karyopharm announced its entry into an agreement with Biogen to acquire Karyopharm’s investigational oral SINE compound KPT-350 targeting certain neurological and neurodegenerative conditions, including amyotrophic lateral sclerosis (ALS). The transaction carries a total deal value of up to $217 million, plus royalties.

- **Exclusive License Agreement with Ono Pharmaceutical Co., Ltd. (Ono) to Develop and Commercialize Selinexor and Eltanexor 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 eltanexor. The agreement includes the development of selinexor and eltanexor 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 carries a total deal value of up to $193.0 million based on the exchange rate on the effective date of the license agreement plus royalties. Karyopharm retains all rights to selinexor and eltanexor outside the Territory.

Selinexor in Multiple Myeloma

- **Ongoing Phase 2b STORM Study Expansion in Patients with Penta-refractory MM.** Karyopharm expects to report top-line data from the expanded STORM study cohort at the end of April 2018, and, assuming a positive outcome, intends to use the data from this study to support a request for accelerated approval from the FDA and conditional approval from the EMA for oral selinexor for penta-refractory MM. The Phase 2b STORM study was previously expanded to include 122 additional patients with penta-refractory MM.

- **Pivotal Phase 3 BOSTON Study Underway.** Karyopharm’s pivotal, randomized Phase 3 BOSTON (**Bo**rtezomib, **S**elinexor and dexame**t**has**o**ne) study is now underway and enrolling patients in 14 countries globally. BOSTON is designed to evaluate 100mg of selinexor dosed once weekly in combination with the proteasome inhibitor Velcade (once weekly) and dexamethasone (SVd), compared to standard twice weekly Velcade 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 and overall response rate. Both the trial design and endpoints have been agreed to by the FDA and the EMA as acceptable to support an application for approval. The Company expects to enroll approximately 360 patients at over 100 clinical sites internationally and is expecting completion of enrollment in 2018, with top-line data anticipated in 2019.

- **Positive Phase 1b/2 STOMP Data Presented at ASH 2017.** Data presentations featuring clinical results from four treatment arms of the ongoing Phase 1b/2 STOMP study evaluating selinexor in combination with standard therapies for the treatment of patients with MM were presented at the American Society of Hematology 2017 Annual Meeting (ASH 2017). Collectively, the STOMP study data presented at ASH 2017 continue to provide evidence of tolerability and robust anti-myeloma activity when selinexor is combined with the currently available standard myeloma therapies, including proteasome inhibitors including Velcade, immunomodulatory drugs and anti-CD38 monoclonal antibodies. Additional treatment arms with selinexor in patients with newly diagnosed disease in combination with lenalidomide, and in combination with the proteasome inhibitor Kyprolis® (carfilzomib) have been added.

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 DLBCL. 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 by the end 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 from the FDA and conditional approval from the EMA 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.** Karyopharm previously 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. The Phase 3 portion is underway and, assuming a positive outcome on the primary end point of progression free survival, the Company intends to use the data from the Phase 2/3 SEAL study to support a New Drug Application in the U.S. and a Marketing Authorization Application (MAA) in Europe for oral selinexor as a potential new treatment for patients with advanced unresectable dedifferentiated liposarcoma. Top-line data from the Phase 3 portion of the SEAL study are anticipated by the end of 2019.

- **Initiation of Investigator Sponsored Phase 2/3 Trial as Maintenance Therapy in Endometrial Cancer Underway.** A randomized Phase 2/3 study of selinexor vs. placebo as maintenance therapy in patients with 1-2 prior platinum-based treatments for advanced endometrial cancer lead by Dr. Ignace Vergote, Head of the Department of Obstetrics and Gynaecology and Gynaecologic Oncology at the Catholic University of Leuven, Belgium, has been initiated.

Eltanexor

- **Positive Phase 1/2 Eltanexor Data Presented at ASH 2017.** Data from a Phase 1/2 study evaluating oral eltanexor in 34 patients with heavily pretreated MM was also presented at ASH 2017. The data showed that eltanexor, both alone or in combination with low-dose dexamethasone, induced responses or disease control and was associated with prolonged survival. The study has been expanded to evaluate eltanexor in patients with advanced colorectal cancer (CRC), castration-resistant prostate cancer (crPC), and myelodysplastic syndrome (MDS). These are indications where selinexor and XPO1 inhibition has shown clear activity, but where the reduced side effects of eltanexor may permit more extended dosing.

Fourth Quarter and Year Ended December 31, 2017 Financial Results

Cash, cash equivalents and investments as of December 31, 2017, including restricted cash, totaled $176.4 million, compared to $175.5 million as of December 31, 2016.

For the year ended December 31, 2017, research and development expense was $107.3 million compared to $86.9 million for the year ended December 31, 2016. For the year ended December 31, 2017, general and administrative expense was $24.9 million compared to $23.9 million for the year ended December 31, 2016.

Karyopharm reported a net loss of $129.0 million, or $2.81 per share, for the year ended December 31, 2017, compared to a net loss of $109.6 million, or $2.92 per share, for the year ended December 31, 2016. Net loss includes stock-based compensation expense of $20.4 million and $22.3 million for the years ended December 31, 2017 and December 31, 2016, respectively.

For the quarter ended December 31, 2017, research and development expense was $34.8 million compared to $20.7 million for the quarter ended December 31, 2016. The increase in research and development expenses resulted primarily from increased expenses on our late stage clinical trials for selinexor and from payments of a portion of the upfront fees we received under our license agreement with Ono. For the quarter ended December 31, 2017, general and administrative expense was $6.2 million compared to $6.5 million for the quarter ended December 31, 2016. Karyopharm reported a net loss of $39.0 million, or $0.80 per share for the quarter ended December 31, 2017, compared to a net loss of $26.9 million, or $0.65 per share, for the quarter ended December 31, 2016. Net loss includes stock-based compensation expense of $4.5 million and $5.1 million for the quarters ended December 31, 2017 and December 31, 2016, respectively.

Financial Outlook

Based on current operating plans, Karyopharm expects that its existing cash, cash equivalents and investments will be sufficient to fund its operations through at least the first quarter of 2019. These plans include the continued clinical development of selinexor in the Company's lead indications with a focus on filing an NDA 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 the United States. Additional key milestones expected in 2018 include a potential MAA filing to the EMA requesting conditional approval for selinexor in MM, topline data from the SADAL study and completion of enrollment in the Phase 3 BOSTON study.

Conference Call Information

Karyopharm will host a conference call today, Thursday, March 15, 2018, at 8:30 a.m. Eastern Time, to discuss the fourth quarter and full year 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: 1181109. 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 standard 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 four 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 enrollment of certain trials, the reporting of data from such trials and potential regulatory filings, the potential to receive milestone and royalty payments under the arrangements with Ono and Biogen 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), eltanexor (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 and the potential future implications of such agreement; the impact of volatility in currency exchange rates; the ability of Karyopharm or Biogen to fully perform their respective obligations under their agreement and the potential future implications of such agreement; 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, 2017, which was filed with the Securities and Exchange Commission (SEC) on November 2, 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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Karyopharm Therapeutics Inc.
Consolidated Balance Sheets

(in thousands, except share and per share amounts)

(Unaudited)

	December 31, 2017	December 31, 2016
ASSETS		
Current assets:		

Cash and cash equivalents	$ 68,997	$ 49,663
Short-term investments	77,472	79,889
Prepaid expenses and other current assets	1,754	2,084
Restricted cash	200	—
Total current assets	148,423	131,636
Property and equipment, net	2,185	2,836
Long-term investments	29,396	45,434
Restricted cash	290	479
Total assets	$ 180,294	$ 180,385
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 5,665	$ 4,751
Accrued expenses	21,445	11,362
Deferred revenue	21,921	—
Deferred rent	303	280
Other current liabilities	133	83
Total current liabilities	49,467	16,476
Deferred rent, net of current portion	1,363	1,666
Total liabilities	50,830	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; 49,533,150 and 41,887,829 shares issued and outstanding at December 31, 2017 and 2016, respectively	5	4
Additional paid-in capital	625,017	528,617
Accumulated other comprehensive loss	(217)	(274)
Accumulated deficit	(495,341)	(366,104)
Total stockholders' equity	129,464	162,243
Total liabilities and stockholders' equity	$ 180,294	$ 180,385

Karyopharm Therapeutics Inc.
Consolidated Statements of Operations

(in thousands, except share and per share amounts)

(Unaudited)

	(Unaudited) For the Quarter Ended, December 31,		For the Year Ended December 31,	
	2017	2016	2017	2016
License and other revenue	$ 1,534	$ 47	$ 1,605	$ 154
Operating expenses:				
Research and development	34,833	20,671	107,273	86,938
General and administrative	6,153	6,541	24,870	23,948
Total operating expenses	40,986	27,212	132,143	110,886
Loss from operations	(39,452)	(27,165)	(130,538)	(110,732)
Other income (expense):				
Interest income	432	358	1,698	1,284
Other income (expense)	(11)	11	(81)	10
Total other income, net	421	369	1,617	1,294
Loss before income taxes	(39,031)	(26,796)	(128,921)	(109,438)
Provision for income taxes	(9)	(139)	(63)	(139)
Net loss	$ (39,040)	$ (26,935)	$ (128,984)	$ (109,577)
Net loss per share—basic and diluted	$ (0.80)	$ (0.65)	$ (2.81)	$ (2.92)
Weighted-average number of common shares outstanding used in net loss per share—basic and diluted	48,644,578	41,376,022	45,899,784	37,523,051