Karyopharm Reports First Quarter 2017 Financial Results and Highlights Recent Progress

− Pivotal Phase 3 BOSTON Study Expected to Commence in May 2017 —
− Secured $52.2 Million Through Recent Equity Financing and ATM Facility —
− Conference Call Scheduled for Today at 8:30 a.m. ET −

NEWTON, Mass., May 04, 2017 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a clinical-stage pharmaceutical company, today reported financial results for the first 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).

"It's been a highly active early 2017 for Karyopharm, marked most notably by establishment of a planned approval path for selinexor in relapsed or refractory diffuse large B-cell lymphoma (DLBCL), our second lead indication after multiple myeloma (MM), following the presentation of robust interim data from the Phase 2b SADAL study at the American Association for Cancer Research (AACR) 2017 Annual Meeting," said Michael G. Kauffman, MD, PhD, Chief Executive Officer of Karyopharm. "After the observation of a 28.6% overall response rate (ORR) with over 7 months median duration of response (DOR), we consulted with the U.S. Food and Drug Administration (FDA) and obtained their agreement to amend the SADAL study to focus solely on the 60mg twice weekly treatment cohort, in which we plan to enroll up to 90 more patients. Assuming we continue to see the response rate and durability observed to date, we plan to use the data from the SADAL study to support a request for accelerated approval in DLBCL. Looking ahead, we remain focused on the initiation of the pivotal Phase 3 BOSTON study where we will evaluate selinexor in combination with Velcade® (bortezomib) and dexamethasone in patients with myeloma previously treated with one to three regimens, moving selinexor into much earlier lines of treatment."

Dr. Kauffman continued, "Importantly, during April 2017, we strengthened our balance sheet by raising net proceeds of approximately $52.2 million in equity financings, including approximately $37.8 million in an underwritten public offering and $14.5 million through our at-the-market (ATM) offering program. We plan to use these funds to support the continued clinical development of selinexor in our lead indications, including in multiple myeloma, DLBCL and other oncology indications, with a focus on filing for accelerated approvals for both myeloma and DLBCL during 2018. In addition, we expect this capital will fund our operations into 2019, while we are preparing to establish a commercial infrastructure for the potential launch of selinexor in North America and Western Europe."

First Quarter 2017 and Recent Events, Highlights and Milestones:

Selinexor in Multiple Myeloma (MM)

- Upcoming Initiation of Pivotal Phase 3 BOSTON Study. Based on the strong combination data recently reported from the Phase 1b STOMP study, Karyopharm plans to initiate a pivotal randomized Phase 3 study, known as the BOSTON (Bortezomib, Selinexor and dexamethasone) study, which will evaluate selinexor in combination with Velcade and dexamethasone (SVd), compared to Velcade and low-dose dexamethasone (Vd) in patients with MM who have had one to three prior lines of therapy. The BOSTON study is expected to enroll approximately 360 patients and commence in May 2017.
- Ongoing Phase 2b STORM Study Expansion in Patients with Penta-refractory MM. The Company has expanded the Phase 2b STORM study, which is expected to include 122 additional patients with penta-refractory MM, a growing unmet medical need in which there are no approved therapies available. Karyopharm expects to report top-line data from the expanded cohort in early 2018, and, assuming a positive outcome, intends to use the expanded STORM study data to support a request for accelerated approval for selinexor in MM.
- Completed Enrollment in Phase 1b/2 STOMP Arm Evaluating Selinexor in Combination with Velcade. In February 2017, Karyopharm completed enrollment in the Phase 1b/2 STOMP arm designed to evaluate selinexor in combination with the proteasome inhibitor Velcade and low-dose dexamethasone (SVd) in heavily pretreated patients with MM. The SVd arm of the STOMP study enrolled 42 patients and the Company expects to report updated data toward the end of 2017.
- Upcoming Initiation of New Phase 1b/2 STOMP Expansion Arm Evaluating Selinexor in Combination with Darzalex® (daratumumab). Karyopharm expects to dose the first patient in a new Phase 1b/2 STOMP expansion 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 approximately 44 patients and the Company expects to report top-line data in late 2017 or early 2018.
- Presented an Overview of Clinical Data Demonstrating Selinexor Activity in Combination with Proteasome Inhibitors and Immunomodulatory Agents. In an oral presentation at the International Myeloma Workshop 2017 annual meeting held March 1-4, 2017 in New Delhi, India, Karyopharm researchers presented an overview of clinical data demonstrating selinexor's activity in combination with proteasome inhibitors and immunomodulatory drugs for the treatment of relapsed or refractory MM.

Selinexor in Diffuse Large B-Cell Lymphoma (DLBCL)

- Top-line Data from Phase 2b SADAL Study in DLBCL Presented in a Late-Breaking Poster at AACR 2017. At the April AACR 2017 Annual Meeting, a late-breaking poster was presented that highlighted top-line data from the Company's ongoing Phase 2b SADAL study evaluating 60mg and 100mg doses of single-agent selinexor in patients with relapsed or refractory DLBCL. The data demonstrated that selinexor achieved an ORR of 28.6% in the first 63 patients, as adjudicated by an independent central radiological committee, and a disease control rate (DCR) of 42.9%. The median overall survival (OS) was 8 months for all patients, consistent with published data in this population. As of the data cutoff date, median survival for the responders had not been reached and is over 9 months. The median DOR across all patients was greater than 7 months with most responses occurring at the first response evaluation (~2 months). As of the data cutoff date, 9 patients who responded remained on treatment, including 6 patients with a complete response (CR). Selinexor showed similar activity against GCB and non-GCB subtypes of DLBCL: Of the 32 patients with DLBCL of the GCB-subtype, selinexor achieved an ORR of 25.0% and DCR of 43.8%. Of the 31 patients with DLBCL of the non-GCB-subtype (ABC), selinexor achieved an ORR of 32.3% and DCR of 41.9%. Among the 72 patients evaluated for safety, the most common adverse events (AEs) across both dosing groups were fatigue (65%), thrombocytopenia (54%), nausea (51%), anorexia (49%), vomiting (35%) and anemia (32%), and were primarily grades 1 and 2 and were managed with dose modifications and/or standard supportive care. As expected, the most common grade 3 and 4 AEs in the 60mg arm were thrombocytopenia (32%), neutropenia (16%), anemia (14%), and fatigue (11%) and were manageable with dose modifications and/or standard supportive care.

As a result of these findings, and in consultation with the FDA, Karyopharm is amending the SADAL study protocol to become a single-arm

study focusing solely on single-agent selinexor dosed at 60mg twice weekly, eliminating the 100mg arm. The study is also being amended to reduce the 14-week treatment-free period to 8 weeks in patients who achieved at least a partial response (PR) on their most recent therapy. Patients whose disease was refractory or did not achieve at least a PR on their prior therapy will continue with the 14-week treatment-free period. The FDA agreed that the modification to a single-arm study was reasonable and that the proposed trial design and indication appear appropriate for accelerated approval, though eligibility for accelerated approval will depend on the complete trial results and available therapies at the time of regulatory action. The Company plans to enroll up to an additional 90 patients to the 60mg single-arm cohort and expects to report top-line results from the SADAL study in mid-2018.

Selinexor in Other Hematologic Malignancies

- Announced Outcome of Phase 2 SOPRA Interim Analysis; Updated AML Development Strategy. In March 2017, Karyopharm announced the results of the planned interim analysis of the Phase 2 SOPRA study evaluating single-agent selinexor in relapsed or refractory acute myeloid leukemia (AML). In concert with the study's independent Data Safety Monitoring Board (DSMB), the Company determined that the SOPRA study would not reach statistical significance for showing superiority of OS on selinexor versus OS on physician's choice (PC), the study's primary endpoint. However, the 13% of selinexor-treated patients who achieved a complete response with or without full hematologic recovery (CR/CRi) showed a substantial OS benefit as compared to PC. As a result, patients were permitted to continue on both the selinexor arm or the PC arm, as applicable, following discussion with their treating physician. Selinexor demonstrated a safety profile consistent with previous studies with similar rates of sepsis and lower rates of febrile neutropenia in the selinexor arm versus the PC arm. Karyopharm plans to continue to explore the use of selinexor in combination with novel and standard agents through investigator-sponsored AML studies.

Selinexor in Solid Tumors

- Completed Enrollment in Phase 2 Portion of the Phase 2/3 SEAL Study. In March 2017, Karyopharm completed enrollment in the Phase 2 portion of the randomized Phase 2/3 SEAL study evaluating single-agent selinexor versus placebo in patients with advanced liposarcoma. Top-line data from the Phase 2 portion of this study are expected in mid-2017. The primary endpoint of the SEAL study is progression free survival (PFS) and both the trial design and endpoints have been agreed to by the FDA and the European Medicines Agency (EMA) as acceptable for approval.
- Oral Presentation Highlighting Efficacy, Safety and Intratumoral Pharmacokinetic Data for Selinexor in Glioblastoma at the 2017 World Federation of Neuro-Oncology Societies (WFNOS). Clinical data from a Phase 2 study evaluating selinexor in patients with recurrent glioblastoma will be highlighted in an oral presentation on May 6, 2017 at WFNOS 2017 by Andrew Lassman, MD, Columbia University Medical Center. These data demonstrate 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 80mg once weekly were as high or higher than those observed with more intensive dosing, and tolerability was improved. Accrual in this Phase 2 study utilizing once weekly dosing continues.

Verdinexor

- Signed Global License Agreement with Anivive Lifesciences for Verdinexor for Animal Health Applications. Earlier this week, Karyopharm and Anivive, a privately-held biotech company, announced their entry into a licensing agreement whereby 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 will make a one-time upfront payment of $1 million to Karyopharm. Anivive agreed to pay up to an additional $43.5 million in certain regulatory, clinical and commercial milestones, assuming approval in both the United States (US) and the European Union (EU). In addition, Anivive agreed to pay Karyopharm a low double-digit royalty on future net sales.

KPT-9274

- Preclinical Efficacy Highlighting KPT-9274's Anti-Cancer Activity in Dogs with Spontaneous Lymphomas Presented as a Late-Breaking Poster at AACR 2017 Annual Meeting. At the April AACR 2017 Annual Meeting, 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 in mid-2017.

Other Corporate and Clinical Developments

- Generated $52.2 Million in Equity Financings. In April 2017, the Company completed the sale of 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 $37.8 million after deducting underwriting discounts and commissions and other estimated offering expenses, and the sale of approximately 1.3 million shares under the ATM offering facility for net proceeds of approximately $14.5 million.

- Partial Clinical Holds Lifted by U.S. FDA. During late March and early April 2017, the FDA's Divisions of Hematology Products, Oncology Products 1 and Oncology Products 2 lifted their respective partial clinical holds placed on the Company's selinexor clinical trials, re-opening enrollment and dosing of new patients in all of the Company's clinical trials across both hematological and solid tumor malignancies. There were no material impacts on development timelines for any of the ongoing selinexor studies.

- Management Change. In April 2017, Justin Renz resigned as the Company's Executive Vice President, Chief Financial Officer and Treasurer to pursue other opportunities. Mr. Renz continues to serve the Company in an advisory capacity in order to ensure a smooth transition. Karyopharm has begun a search process for the selection and appointment of a new Chief Financial Officer. In the interim, Michael Todisco, who serves as the Company's Vice President, Finance, leads the Company's internal finance function.

First Quarter 2017 Financial Results

Cash, cash equivalents and investments as of March 31, 2017, including restricted cash, totaled $150.6 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 estimated offering expenses, were approximately $37.8 million. In addition, during April 2017, the Company completed the sale of approximately 1.3 million shares under the ATM offering facility for net proceeds of approximately $14.5 million.

For the quarter ended March 31, 2017, research and development expense was $24.1 million compared to $21.8 million for the quarter ended March 31, 2016. For the quarter ended March 31, 2017, general and administrative expense was $6.3 million compared to $5.6 million for the quarter ended March 31, 2016.

Karyopharm reported a net loss of $29.9 million, or $0.71 per share, for the quarter ended March 31, 2017, compared to a net loss of $27.1 million, or $0.75 per share, for the quarter ended March 31, 2016. Net loss includes stock-based compensation expense of $5.9 million and $5.2 million for the quarters ended March 31, 2017 and March 31, 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 $85 to 90 million. Based on current operating plans, Karyopharm expects that its existing cash and cash equivalents, along with the $52.2 million of net proceeds raised in April 2017, will be sufficient to fund its research and development programs and operations into 2019, including the continued clinical development of selinexor in our 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, Thursday, May 4, 2017, at 8:30 a.m. Eastern Time, to discuss the first 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: 10603764. 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 2,000 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 multiple myeloma in combination with low-dose dexamethasone (STORM) and backbone therapies (STOMP), 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 May 2017. 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 of certain trials and of the reporting of data from such trials, and Karyopharm's financial outlook. 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 next generation 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; 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, 2016, which was filed with the Securities and Exchange Commission (SEC) on March 16, 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.

Velcade® is a registered trademark of Takeda Pharmaceutical Company Limited
Darzalex® is a registered trademark of Janssen Biotech, Inc.

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

	March 31, 2017	December 31, 2016
Assets		
Current assets:		
Cash and cash equivalents	$ 25,577	$ 49,663

Short-term investments	84,307	79,889
Restricted cash	200	—
Prepaid expenses and other current assets	2,146	2,084
Total current assets	112,230	131,636
Property and equipment, net	2,654	2,836
Long-term investments	40,257	45,434
Restricted cash	279	479
Other assets	15	—
Total assets	$ 155,435	$ 180,385
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 4,245	$ 4,751
Accrued expenses	10,740	11,362
Deferred rent	286	280
Other current liabilities	210	83
Total current liabilities	15,481	16,476
Deferred rent, net of current portion	1,591	1,666
Total liabilities	17,072	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; 41,902,255 and 41,887,829 shares issued and outstanding at March 31, 2017 and December 31, 2016, respectively	4	4
Additional paid-in capital	534,838	528,617
Accumulated other comprehensive loss	(204)	(274)
Accumulated deficit	(396,275)	(366,104)
Total stockholders' equity	138,363	162,243
Total liabilities and stockholders' equity	$ 155,435	$ 180,385

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

	Three Months Ended, March 31,	
	2017	2016
Contract and grant revenue	$ 68	$ —
Operating expenses:		
Research and development	24,083	21,795
General and administrative	6,264	5,554
Total operating expenses	30,347	27,349
Loss from operations	(30,279)	(27,349)
Other income (expense):		
Interest income	400	286
Other income (expense)	(15)	4
Total other income (expense), net.	385	290
Loss before income taxes	(29,894)	(27,059)
Provision for income taxes	(23)	—
Net loss	$ (29,917)	$ (27,059)
Net loss per share—basic and diluted	$ (0.71)	$ (0.75)
Weighted-average number of common shares outstanding used in net loss per share—basic and diluted	41,894,796	35,878,502

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