Karyopharm Reports First Quarter 2018 Financial Results and Highlights Recent Progress

May 10, 2018

-- Oral selinexor Achieves 25.4% Overall Response Rate and Median Duration of Response of 4.4 Months in Patients with Penta-Refractory Myeloma --

-- Completed $155.3 Million Public Equity Offering, Including Full Exercise of Underwriters Option to Purchase Additional Shares --

-- Company Plans to Submit NDA Requesting Accelerated Approval for Selinexor in Penta-Refractory Myeloma During Second Half of 2018 --

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

NEWTON, Mass., May 10, 2018 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a clinical-stage pharmaceutical company, today reported financial results for the first quarter 2018 and provided an overview of recent accomplishments and clinical development plans for selinexor, its lead, novel, oral SINE compound, eltanexor, its second-generation oral SINE compound, and KPT-9274 its novel, oral, dual inhibitor of PAK4 and NAMPT.

"The positive top-line data recently reported from the Phase 2b STORM study evaluating selinexor in patients with penta-refractory myeloma are an important step forward toward the approval of selinexor," said Michael G. Kauffman, MD, PhD, Chief Executive Officer of Karyopharm. "Our progress is further enabled by the successful completion of a $155.3 million equity financing and the receipt of Fast Track designation from the U.S. Food and Drug Administration (FDA) for selinexor for the treatment of patients with penta-refractory multiple myeloma. We look forward to submitting a New Drug Application (NDA) to the FDA during the second half of 2018, with a request for accelerated approval for selinexor in penta-refractory myeloma, followed thereafter by a Marketing Authorization Application (MAA) submission to the European Medicines Agency (EMA) in early 2019, with a request for conditional approval in the same indication."

First Quarter 2018 and Recent Events

Selinexor in Multiple Myeloma

- **Reported Positive Top-line Data from the Phase 2b STORM Study Expansion in Patients with Penta-Refractory Myeloma.** Last week, Karyopharm reported positive top-line results from the Phase 2b STORM study evaluating selinexor in heavily pretreated patients with refractory multiple myeloma. For the STORM study's primary objective, oral selinexor achieved a 25.4% ORR, which included two stringent complete responses (CRs) and 29 partial (PRs) or very good partial responses (VGPRs). One of the stringent CRs was negative for minimal residual disease (MRD), highly significant in this penta-refractory population. The median DOR, a key secondary objective, was 4.4 months. Oral selinexor demonstrated a predictable and manageable tolerability profile. Safety results were consistent with those previously reported from Part 1 of this study and from other selinexor studies and no new safety signals were identified. Karyopharm plans to submit detailed STORM study results for presentation at an upcoming medical oncology meeting.

- **Selinexor Receives Fast Track Designation from FDA for the Treatment of Patients with Penta-Refractory Myeloma.** In addition to Orphan Drug Designation, selinexor was recently granted Fast Track designation by the FDA for the treatment of patients with penta-refractory multiple myeloma. The FDA's stated indication is consistent with the design of Karyopharm's Phase 2b STORM study.

- **On Track to Submit NDA for Selinexor in Penta-Refractory Myeloma.** Karyopharm plans to submit an NDA to the FDA during the second half of 2018, with a request for accelerated approval for oral selinexor as a new treatment for patients with penta-refractory multiple myeloma. The Company also plans to submit an MAA to the EMA in early 2019 with a request for conditional approval for selinexor in the same indication.

- **Pivotal Phase 3 BOSTON Study Underway.** Karyopharm's pivotal, randomized Phase 3 BOSTON study is now well underway and enrolling patients in 14 countries globally. BOSTON is evaluating 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 multiple myeloma who have had one to three prior lines of therapy. The primary endpoints of the study are progression free survival and overall response rate. Assuming a positive outcome, the data from the BOSTON study will be used to support regulatory submissions to the FDA and EMA for full approvals. The Company expects to enroll approximately 360 patients at over 100 clinical sites internationally and expects to complete enrollment by the end of 2018, with top-line data anticipated in 2019.

Selinexor in Diffuse Large B-Cell Lymphoma (DLBCL)

- **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 who received 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 positive results 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 SEAL study to support an NDA and an MAA submission for oral selinexor 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.

- **Ongoing Investigator Sponsored Phase 2/3 Trial as Maintenance Therapy in Endometrial Cancer Underway.** A randomized Phase 2/3 study of selinexor versus placebo as maintenance therapy in patients with one or two 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, is currently ongoing.

AACR 2018

- **Eight Preclinical Data Posters Presented at the American Association for Cancer Research (AACR) 2018 Annual Meeting.** In March 2018, eight posters were presented at AACR 2018 which featured preclinical data for selinexor, eltanexor and KPT-9274. Collectively, these data continue to provide important insights that Karyopharm believes will help guide the future clinical development of all three product candidates across a wide range of malignancies, including ovarian, prostate and pancreatic cancers and neuroblastoma.

Corporate and Financial

- **Strengthened the Balance Sheet with a Public Equity Offering.** On May 7, 2018, Karyopharm completed an underwritten public offering of 10,525,424 shares of its common stock at a price to the public of $14.75 per share. The gross proceeds to Karyopharm from the offering were $155.3 million. After deducting the underwriting discounts and commissions and other estimated offering expenses the net proceeds were $145.6 million.

- **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 for Biogen's acquisition of 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.

First Quarter Ended March 31, 2018 Financial Results

Cash, cash equivalents and investments as of March 31, 2018, including restricted cash, totaled $141.5 million, compared to $176.4 million as of December 31, 2017.

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For the quarter ended March 31, 2018, Karyopharm recognized $10.0 million in revenue, compared to $0.1 million for the three months ended March 31, 2017. The increase in revenue was the result of the upfront payment received from the asset sale of KPT-350 to Biogen in January 2018.

For the quarter ended March 31, 2018, research and development expense was $41.3 million compared to $24.1 million for the quarter ended March 31, 2017. For the quarter ended March 31, 2018, general and administrative expense was $7.6 million compared to $6.3 million for the quarter ended March 31, 2017.

Karyopharm reported a net loss of $38.5 million, or $0.78 per share, for the quarter ended March 31, 2018, compared to a net loss of $29.9 million, or $0.71 per share, for the quarter ended March 31, 2017. Net loss includes stock-based compensation expense of $4.2 million and $5.9 million for the quarters ended March 31, 2018 and March 31, 2017, respectively.

Financial Outlook

Karyopharm expects its operating cash burn, including research and development and general and administrative expenses, for the year ending December 31, 2018 to be in the range of $175 to 185 million. Based on current operating plans, Karyopharm expects that its existing cash, cash equivalents and investments will be sufficient to fund its operations into the third 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 multiple myeloma during 2018 and preparing the commercial infrastructure and hiring a sales force for the potential launch of selinexor in the U.S. Additional key milestones expected in 2018 include preparing for a potential MAA submission to the EMA requesting conditional approval for selinexor in multiple myeloma, topline data from the SADAL study and completion of enrollment in the Phase 3 BOSTON study.

Further Information About Potential Accelerated Approval for Selinexor in Multiple Myeloma

The FDA instituted its Accelerated Approval Program to allow for expedited approval of drugs that treat serious conditions and that fill an unmet medical need based on a surrogate endpoint or an intermediate clinical endpoint thought to predict clinical benefit, like ORR. Accelerated approval is available only for drugs that provide a meaningful therapeutic benefit over existing treatments at the time of consideration of the application for accelerated approval, which the FDA has recently reiterated in its feedback to the Company. Particularly in disease areas with multiple available and potential new therapies, such as multiple myeloma, accelerated approval carries a high regulatory threshold. Consistent with its general guidance, the FDA has noted to the Company its preference for randomized studies geared toward full approval, which the Company has undertaken with the pivotal, Phase 3 BOSTON study, and has reminded the Company that accelerated approval requires patients to have exhausted approved therapies. The Company recently received Fast Track designation for selinexor for the treatment of patients with penta-refractory myeloma from the FDA, which is available to therapeutics treating an unmet medical need in a serious condition. In light of this recognition that the STORM patient population represents an unmet medical need and the positive top-line data reported previously, the Company believes that the STORM study should support its request to the FDA for accelerated approval.

Conference Call Information

Karyopharm will host a conference call today, Thursday, May 10, 2018, at 8:30 a.m. Eastern Time, to discuss the first quarter 2018 financial results, recent accomplishments, clinical developments and business plans. To access the conference call, please dial (855) 437-4406 (local) or (484) 756-4292 (international) at least 10 minutes prior to the start time and refer to conference ID 6798355. A live audio webcast of the call will be available under "Events & Presentations" in the Investor section of the Company's website, http://investors.karyopharm.com/events-presentations. An archived webcast will be available on the Company's website 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 2,400 patients have been treated with selinexor. In April 2018, Karyopharm reported positive top-line data from the Phase 2b STORM study evaluating selinexor in combination with low-dose dexamethasone in patients with penta-refractory multiple myeloma. Selinexor has been granted Orphan Drug Designation in multiple myeloma and Fast Track designation for the patient population evaluated in the STORM study. Karyopharm plans to submit a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) during the second half of 2018, with a request for accelerated approval for oral selinexor as a new treatment for patients with penta-refractory multiple myeloma. The Company also plans to submit a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) in early 2019 with a request for conditional approval. Selinexor is also being evaluated in several other 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) and as a potential backbone therapy in combination with approved therapies (STOMP), and in diffuse large B-cell lymphoma (SADAL), liposarcoma (SEAL), and an investigator-sponsored study in endometrial cancer (SIENDO), 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 Karyopharm'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 timing of submissions to regulatory authorities and the potential availability of accelerated approval pathways, 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 the impact on potential regulatory filings, the potential to receive milestone and royalty payments under third party arrangements 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 regulators will agree that selinexor qualifies for accelerated approval in the U.S. or conditional approval in the E.U. as a result of the data from the STORM study in patients with penta-refractory myeloma or that any of Karyopharm's drug candidates, 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 its third party collaborators or successors in interest to fully perform their respective obligations under collaboration or license agreements and the potential future implications of such agreements; 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, 2017, which was filed with the Securities and Exchange Commission (SEC) on March 15, 2018, 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.
CONDENSED CONSOLIDATED BALANCE SHEETS
(unaudited)
(in thousands, except share and per share amounts)

	March 31, 2018	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$ 37,499	$ 68,997
Short-term investments	93,418	77,472
Prepaid expenses and other current assets	2,396	1,754
Restricted cash	—	200
Total current assets	133,313	148,423
Property and equipment, net	2,454	2,185
Long-term investments	10,314	29,396
Restricted cash	292	290
Total assets	$ 146,373	$ 180,294
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 4,949	$ 5,665
Accrued expenses	21,545	21,445
Deferred revenue	19,729	21,921
Deferred rent	178	303
Other current liabilities	333	133
Total current liabilities	46,734	49,467
Deferred revenue, net of current portion	2,192	—
Deferred rent, net of current portion	1,918	1,363
Total liabilities	50,844	50,830
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; 49,670,328 and 49,533,150 shares issued and outstanding at March 31, 2018 and December 31, 2017, respectively	5	5

Additional paid-in capital	629,610	625,017
Accumulated other comprehensive loss	(286)	(217)
Accumulated deficit	(533,800)	(495,341)
Total stockholders' equity	95,529	129,464
Total liabilities and stockholders' equity	$ 146,373	$ 180,294

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

	Three Months Ended March 31,	
	2018	**2017**
License and other revenue	$ 10,000	$ 68
Operating expenses:		
Research and development	41,321	24,083
General and administrative	7,621	6,264
Total operating expenses	48,942	30,347
Loss from operations	(38,942)	(30,279)
Other income (expense):		
Interest income	509	400
Other expense	(14)	(15)
Total other income, net	495	385
Loss before income taxes	(38,447)	(29,894)
Provision for income taxes	(12)	(23)
Net loss	$ (38,459)	$ (29,917)
Net loss per share—basic and diluted	$ (0.78)	$ (0.71)
Weighted-average number of common shares outstanding used in net loss per share—basic and diluted	49,602,809	41,894,796

Primary Logo

Source: Karyopharm Therapeutics Inc.