Karyopharm Reports Second Quarter 2019 Financial Results and Highlights Recent Company Progress

− XPOVIO™ Commercial Launch Underway Following July 3rd FDA Accelerated Approval −
− XPOVIO is the First and Only Prescription Medicine Approved in the U.S. for the Treatment of Patients with Multiple Myeloma whose Disease is Refractory to Proteasome Inhibitors, Immunomodulatory Agents, and an Anti-CD38 Monoclonal Antibody –
− Conference Call Scheduled for Today at 8:30 a.m. ET −

NEWTON, Mass., Aug. 06, 2019 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), an oncology-focused pharmaceutical company, today reported financial results for the second quarter 2019. In addition, Karyopharm highlighted select corporate milestones, including an update regarding the initial commercial launch of XPOVIO, and provided an overview of its key clinical development programs.

"Our second quarter progress was followed by a transformational milestone: the U.S. Food and Drug Administration (FDA) granting accelerated approval of oral XPOVIO™ (www.XPOVIO.com), indicated for patients with heavily pre-treated multiple myeloma," said Michael G. Kauffman, MD, PhD, Chief Executive Officer of Karyopharm. "The commercial launch of XPOVIO, our first approved product, is off to a strong start with encouraging early prescribing trends from both academic and community-based physicians throughout the U.S. While we are laser-focused on the success of the initial launch, we remain deeply committed to serving the future needs of patients well beyond those directly indicated in the U.S. accelerated approval. To that end, we eagerly await the clinical trial results from the ongoing Phase 3 BOSTON study and continue to support the evaluation of a Marketing Authorization Application (MAA) of selinexor currently under review by the European Medicines Agency (EMA). And finally, we expect to rapidly advance the regulatory filings for selinexor in both the U.S. and Europe requesting accelerated and conditional approval, respectively, for patients with relapsed or refractory diffuse large B-cell lymphoma."

Second Quarter 2019 Highlights and Recent Progress

Selinexor in Multiple Myeloma

- XPOVIO™ (selinexor) Receives Accelerated Approval from the FDA. On July 3, 2019, the FDA approved oral XPOVIO, Karyopharm's first-in-class, nuclear export inhibitor. XPOVIO was approved in combination with dexamethasone for the treatment of adult patients with relapsed or refractory multiple myeloma who have received at least four prior therapies and whose disease is refractory to at least two proteasome inhibitors, at least two immunomodulatory agents, and an anti-CD38 monoclonal antibody. XPOVIO is the first of a novel drug class designated selective inhibitors of nuclear export (SINE) and is the first ever nuclear export inhibitor approved for human use. The first indication is approved under accelerated approval based on response rate. The ongoing Phase 3 BOSTON study will serve as the confirmatory trial for the accelerated approval of XPOVIO. As with all accelerated approvals, continued approval the treatment of myeloma may be contingent upon verification and description of clinical benefit in a confirmatory trial.

- XPOVIO Commercial Launch Underway in the U.S. On July 9, 2019, XPOVIO became commercially available to patients in the U.S. The commercial launch of XPOVIO is being supported by approximately 70 Karyopharm sales representatives and nurse liaisons as well as an extensive patient and healthcare provider support program called KaryForwardTM. Karyopharm's commercial efforts are also being supplemented by patient support initiatives coordinated by our dedicated network of participating specialty pharmacy providers. Early prescribing trends are encouraging with robust demand from both academic and community-based physicians throughout the U.S. with early prescriptions being filled for patients with Medicare and commercial insurance coverage.

- European Medicines Agency (EMA) Validates Marketing Authorization Application (MAA). In January 2019, Karyopharm submitted a MAA to the EMA requesting conditional approval for selinexor, in combination with dexamethasone, as a new treatment for patients with heavily pretreated multiple myeloma based on the results of the Phase 2b STORM study. As a customary part of the MAA review process, Karyopharm received the consolidated list of questions from EMA in early May 2019 and received additional feedback, including the integrated inspection report, based on routine site audits and other activities. The Company promptly addressed the questions and feedback with EMA and the evaluation process of the MAA is ongoing. The Company expects to receive a decision on the application by the end of 2019 or early 2020.

- Pivotal Phase 3 BOSTON Study in Progress. Karyopharm's pivotal, randomized Phase 3 BOSTON study is progressing and patient enrollment is complete. Top-line data are expected by the end of 2019 or early 2020 contingent upon the occurrence of progression-free survival (PFS) events, the primary endpoint of the study. The BOSTON study is evaluating 100mg of selinexor dosed *once* weekly in combination with the proteasome inhibitor Velcade® (bortezomib) (*once* weekly) and low dose 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. Data from the BOSTON study, if positive, is expected to be used to support regulatory submissions to the FDA and EMA requesting the use of selinexor in patients with multiple myeloma who have received at least one prior therapy.

- New and Updated Phase 1b/2 STOMP Data Presented at European Hematology Association (EHA) 2019 Annual Meeting. Three selinexor abstracts highlighting new and updated clinical data from patients receiving a combination of selinexor and standard of care myeloma drugs were presented in June at the EHA 2019 Annual Meeting. Specifically, clinical data from the Kyprolis® (carfilzomib), Darzalex® (daratumumab) and Pomalyst® (pomalidomide) arms of the Phase 1b/2 STOMP study were presented. For the Kyprolis arm, once weekly oral selinexor in combination with low dose dexamethasone demonstrated a 78% overall response rate (ORR) in patients with heavily pre-treated, Kyprolis-naïve multiple myeloma. For the Darzalex arm, once weekly oral selinexor in combination with low dose dexamethasone demonstrated a 73% ORR in patients with heavily pre-treated, Darzalex-naïve multiple myeloma. And finally, for the Pomalyst arm, once weekly oral selinexor in combination with low dose dexamethasone demonstrated a 57% ORR in patients with Revlimid® (lenalidomide)-relapsed or -refractory, Pomalyst-naïve multiple myeloma with PFS of 12.2 months. Across all 3 arms of the STOMP study presented, the most common treatment-related adverse events (AEs) were cytopenias, along with gastrointestinal and constitutional symptoms; most were manageable with dose modifications and/or standard supportive care.

Selinexor in Diffuse Large B-Cell Lymphoma (DLBCL)

- NDA and MAA Expected to be Submitted between Q4 2019 and Q1 2020. Following the positive results from the Phase 2b SADAL study that were first presented at the America Society of Hematology 2018 Annual Meeting and then updated in June at the 2019 International Conference on Malignant Lymphoma, Karyopharm expects to submit a New Drug Application (NDA) to the FDA and an MAA to the EMA requesting accelerated and conditional approval of selinexor, respectively, as a treatment for patients with relapsed or refractory DLBCL after at least two prior multi-agent therapies and who are ineligible for stem cell transplantation including CAR-T (chimeric antigen receptor modified T cell) therapy. In addition to Orphan Drug Designation, selinexor was granted Fast Track designation by the FDA in 2018.

Selinexor in Solid Tumors

- Ongoing Phase 3 Portion of the Phase 2/3 SEAL Study in Liposarcoma. Karyopharm previously reported positive results from the Phase 2 portion of the randomized, blinded Phase 2/3 SEAL study evaluating single-agent selinexor versus placebo in patients with previously treated, advanced unresectable dedifferentiated liposarcoma. Enrollment is currently ongoing in the Phase 3 portion of the SEAL study. Assuming a positive outcome on the primary endpoint of PFS, the Company intends to use the data from the SEAL study to support NDA and MAA submissions requesting approval for selinexor for patients with advanced unresectable dedifferentiated

liposarcoma. Top-line data from the Phase 3 portion of the SEAL study are anticipated in 2020.

- Company-Sponsored Phase 3 SIENDO Study Evaluating Selinexor as Maintenance Therapy in Endometrial Cancer Now Underway. During the first quarter of 2019, an Investigational New Drug Application (IND) was submitted and accepted by the FDA for a randomized, blinded Phase 2/3 study evaluating selinexor versus placebo as a maintenance therapy in patients with advanced or recurrent endometrial cancer following one prior platinum-based treatment. There are currently no approved therapies to treat patients with advanced or recurrent endometrial cancer in the maintenance setting. The primary endpoint of the SIENDO study is PFS. This study is being led by Professor Ignace Vergote, MD, PhD, Head of the Department of Obstetrics and Gynaecology and Gynaecologic Oncology at the Catholic University of Leuven, Belgium. Karyopharm is targeting enrollment completion for SIENDO in 2020.

- Updated Phase 2 KING Data in Glioblastoma Presented at American Society of Clinical Oncology (ASCO) 2019 Annual Meeting. An abstract highlighting updated efficacy and safety results from the Phase 2 KING study evaluating single-agent selinexor in patients with recurrent glioblastoma was presented in June at the ASCO 2019 Annual Meeting. The KING study evaluated three different dosing schedules with selinexor (50mg/m2 twice per week, 60mg twice per week and 80mg once per week) and the reported results followed completion of accrual in the non-surgical cohorts in the study (n=68). Based on the efficacy and tolerability results from the study, the 80mg once per week dosing regimen is recommended for further evaluation. Of the 30 patients treated in the 80mg dosing cohort, the overall response rate reported was 10%, with 19% of patients achieving a 6-month PFS rate and 30% of patients achieving a 6-cycle PFS rate. The most common non-hematologic AEs in this cohort were nausea, fatigue, anorexia, and vomiting, all of which were Grade 1 or 2 events. The most common treatment-related hematological AEs were primarily Grade 1 and 2 and included leukopenia, neutropenia, anemia, and thrombocytopenia. There was one case (3%) of Grade 4 treatment-related lymphopenia and no Grade 5 treatment-related AEs were reported.

Corporate Updates

- Founder Sharon Shacham Receives New York Intellectual Property Law Association (NYIPLA) Inventor of the Year Award. In May, Karyopharm's founder, President and Chief Scientific Officer, Sharon Shacham, PhD, MBA, received the esteemed NYIPLA 2019 "Inventor of the Year" award. Dr. Shacham was recognized for her scientific research that led to the development of oral selinexor and related compounds. Past winners of this award have included the inventors of CAR-T therapy, Gleevec®, Valium®, LASIK laser vision correction and Priceline.com, among many others.

Second Quarter 2019 Financial Results

Cash, cash equivalents and investments as of June 30, 2019, including restricted cash, totaled $217.9 million, compared to $330.9 million as of December 31, 2018.

License and other revenue for the quarter ended June 30, 2019 was $9.5 million, compared to $19.9 million for the quarter ended June 30, 2018, both of which were primarily related to the Company's license agreements with Antengene and Ono, respectively.

For the quarter ended June 30, 2019, research and development expense was $26.5 million, compared to $44.7 million for the quarter ended June 30, 2018. Karyopharm expects research and development expense to be relatively consistent for the remainder of 2019 compared to the second quarter of 2019. For the quarter ended June 30, 2019, general and administrative expense was $24.7 million compared to $9.5 million for the quarter ended June 30, 2018. The increase in general and administrative expenses compared to the prior year period was due primarily to the hiring of the Karyopharm commercial team and related commercial launch preparation activities to support the U.S. commercial launch of XPOVIO.

Karyopharm reported a net loss of $43.4 million, or $0.71 per share, for the quarter ended June 30, 2019, compared to a net loss of $33.7 million, or $0.60 per share, for the quarter ended June 30, 2018. Net loss includes non-cash stock-based compensation expense of $4.1 million and $4.4 million for the quarters ended June 30, 2019 and June 30, 2018, respectively.

2019 Financial Outlook

Based on its current operating plans, Karyopharm expects its non-GAAP operating expenses, which excludes stock-based compensation expense, for the full year 2019 to be in the range of $200 million to $215 million. The Company expects that its existing cash, cash equivalents and investments will be sufficient to fund its operations into the second half of 2020. Additional key activities expected in 2019 include supporting the ongoing multiple myeloma regulatory filing for selinexor in Europe, progressing the pivotal Phase 3 BOSTON study in multiple myeloma and potentially submitting an NDA and MAA, in the U.S. and Europe, respectively, in DLBCL.

Non-GAAP Financial Information and Other Disclosures

Karyopharm uses a non-GAAP financial measure, non-GAAP operating expense, to provide operating expense guidance. Karyopharm believes this non-GAAP financial measure is useful to investors because it provides greater transparency regarding Karyopharm's operating performance as it excludes non-cash stock compensation expense. This non-GAAP financial measure should not be considered a substitute or an alternative to GAAP total operating expense and should not be considered a measure of Karyopharm's liquidity. Instead, non-GAAP operating expense should only be used to supplement an understanding of Karyopharm's operating results as reported under GAAP. Karyopharm has not provided GAAP reconciliation for its forward-looking non-GAAP annual operating expense because Karyopharm cannot reliably predict without unreasonable efforts the timing or amount of the factors that substantially contribute to the projection of stock compensation expense, which is excluded from the forward-looking non-GAAP financial measure.

Conference Call Information

Karyopharm will host a conference call today, Tuesday, August 6, 2019, at 8:30 a.m. Eastern Time, to discuss the second quarter 2019 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 5550468. 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.

Important Safety Information

The most common adverse reactions observed in patients treated with XPOVIO (incidence ≥20%) are thrombocytopenia, fatigue, nausea, anemia, decreased appetite, decreased weight, diarrhea, vomiting, hyponatremia, neutropenia, leukopenia, constipation, dyspnea, and upper respiratory tract infection.

The treatment discontinuation rate due to adverse reactions was 27%; 53% of patients had a reduction in the XPOVIO dose, and 65.3% had the dose of XPOVIO interrupted. The most frequent adverse reactions requiring permanent discontinuation in 4% or greater of patients who received XPOVIO included fatigue, nausea, and thrombocytopenia. The rate of fatal adverse reactions was 8.9%.

The full Prescribing Information for XPOVIO is available at www.XPOVIO.com.

About Karyopharm Therapeutics

Karyopharm Therapeutics Inc. (Nasdaq: KPTI) is an oncology-focused pharmaceutical company dedicated to the discovery, development, and commercialization of novel first-in-class drugs directed against nuclear export 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). Karyopharm's lead compound, XPOVIO™ (selinexor), received accelerated approval from the FDA in July 2019 in combination with dexamethasone as a treatment for patients with heavily pretreated multiple myeloma. A Marketing Authorization Application for selinexor is also currently under review by the European Medicines Agency. 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 has several 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 Karyopharm's expectations relating to XPOVIO for the treatment of patients with heavily pretreated multiple myeloma, commercialization of XPOVIO or any of its drug candidates, submissions to, and the review and potential approval of selinexor by, regulatory authorities, including the anticipated timing of such submissions and actions and the potential availability of accelerated approval pathways, and the therapeutic potential of and potential clinical development plans for Karyopharm's drug candidates, especially selinexor. Such statements are subject to numerous important factors, risks and uncertainties, many of which are beyond Karyopharm's control, that may cause actual events or results to differ materially from Karyopharm's current expectations. For example, there can be no guarantee that Karyopharm will successfully commercialize XPOVIO; that regulators will agree that selinexor qualifies for conditional approval in the E.U. as a result of data from the STORM study or accelerated or conditional approval in the U.S. or EU, respectively, based on the SADAL study in patients with relapsed or refractory DLBCL, or that any of Karyopharm's drug candidates, including selinexor, will successfully complete necessary 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: the timing and costs involved in commercializing XPOVIO or any of Karyopharm's drug candidates that receive regulatory approval; the ability to retain regulatory approval of XPOVIO or any of Karyopharm's drug candidates that receive regulatory approval; 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 U.S. Food and Drug Administration 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, 2019, which was filed with the Securities and Exchange Commission (SEC) on May 9, 2019, 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)

	June 30, 2019	December 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	$ 75,545	$ 118,021
Short-term investments	141,614	210,178
Prepaid expenses and other current assets	5,671	6,413
Total current assets	222,830	334,612
Property and equipment, net	3,375	3,863
Operating lease right-of-use assets	11,180	—
Long-term investments	—	2,001
Restricted cash	715	716
Total assets	$ 238,100	$ 341,192

Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 1,113	$ 4,332
Accrued expenses	27,799	32,493
Deferred revenue	1,287	9,362
Operating lease liabilities	1,522	—
Deferred rent	—	390
Other current liabilities	453	327
Total current liabilities	32,174	46,904
Convertible senior notes	106,157	102,664
Operating lease liabilities, net of current portion	14,055	—
Deferred revenue, net of current portion	3,245	4,532
Deferred rent, net of current portion	—	3,922
Total liabilities	155,631	158,022
Stockholders' equity:		
Preferred stock, $0.0001 par value; 5,000,000 shares authorized; none issued and outstanding	—	—
Common stock, $0.0001 par value; 200,000,000 shares authorized; 60,965,505 shares issued and outstanding at June 30, 2019; 100,000,000 shares authorized; 60,829,308 shares issued and outstanding at December 31, 2018	6	6
Additional paid-in capital	865,726	857,156
Accumulated other comprehensive income (loss)	61	(244)
Accumulated deficit	(783,324)	(673,748)
Total stockholders' equity	82,469	183,170
Total liabilities and stockholders' equity	$ 238,100	$ 341,192

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,	
	2019	2018	2019	2018
License and other revenue	$ 9,493	$ 19,891	$ 9,648	$ 29,891
Operating expenses:				
Research and development	26,517	44,734	64,491	86,055
General and administrative	24,662	9,489	51,765	17,110
Total operating expenses	51,179	54,223	116,256	103,165
Loss from operations	(41,686)	(34,332)	(106,608)	(73,274)
Other income (expense):				
Interest income	1,412	653	3,183	1,162
Interest expense	(3,089)	—	(6,087)	—
Other (expense) income	(44)	7	(46)	(7)
Total other (expense) income, net	(1,721)	660	(2,950)	1,155
Loss before income taxes	(43,407)	(33,672)	(109,558)	(72,119)
Income tax (provision) benefit	(8)	17	(18)	5
Net loss	$ (43,415)	$ (33,655)	$ (109,576)	$ (72,114)
Net loss per share—basic and diluted	$ (0.71)	$ (0.60)	$ (1.80)	$ (1.36)
Weighted-average number of common shares outstanding used in net loss per share	60,929,024	56,089,159	60,892,860	52,862,194

—basic and diluted

Source: Karyopharm Therapeutics Inc.

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https://investors.karyopharm.com/2019-08-06-Karyopharm-Reports-Second-Quarter-2019-Financial-Results-and-Highlights-Recent-Company-Progress