# Karyopharm Reports Second Quarter 2018 Financial Results and Highlights Recent Progress

August 7, 2018

*-- Completed Rolling Submission of a New Drug Application to FDA Seeking Accelerated Approval for Selinexor As a New Treatment for Patients with Penta-Refractory Multiple Myeloma --*

*-- On Track to Report Top-Line Phase 2b SADAL Data in DLBCL and Complete Enrollment in the Pivotal Phase 3 BOSTON Study in Multiple Myeloma by the End of 2018 --*

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

NEWTON, Mass., Aug. 07, 2018 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a clinical-stage pharmaceutical company, today reported financial results for the second quarter 2018 and provided an overview of recent accomplishments and clinical development plans for selinexor, its lead, novel, oral SINE compound and its other pipeline programs.

"We have made tremendous progress toward advancing our lead drug candidate, selinexor, as we strive to improve the lives of patients with myeloma and other forms of cancer. Most notably, we have now completed the submission of our first New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for selinexor as a potential new treatment for patients with penta-refractory myeloma," said Michael G. Kauffman, MD, PhD, Chief Executive Officer of Karyopharm. "The positive results from the Phase 2b STORM study announced during the quarter demonstrated that treatment with selinexor may result in an important clinical benefit in this patient population and we look forward to working with the FDA during their review of the application. We are making excellent progress in advancing commercial preparation for the potential launch of selinexor in the U.S., as well as preparing a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA), which we expect to submit in early 2019, with a request for conditional approval, also in penta-refractory myeloma. Finally, while completing our first-ever NDA submission is clearly a landmark event for Karyopharm, we remain committed to fulfilling the full potential of selinexor as we advance our clinical strategy in earlier lines of treatment in myeloma and in additional tumor types."

**Second Quarter 2018 and Recent Events**

*Selinexor in Multiple Myeloma*

- **Submitted NDA in Penta-Refractory Myeloma.** Karyopharm completed its submission of an NDA to the FDA seeking accelerated approval for selinexor, its lead, novel, oral SINE compound, as a new treatment for patients with penta-refractory multiple myeloma. Patients with penta-refractory myeloma have previously received the two proteasome inhibitors (PIs), Velcade® (bortezomib) and Kyprolis® (carfilzomib), the two immunomodulatory drugs (IMiDs), Revlimid® (lenalidomide) and Pomalyst® (pomalidomide), and the anti-CD38 monoclonal antibody Darzalex® (daratumumab) as well as alkylating agents, and their disease is refractory to at least one PI, at least one IMiD, Darzalex and their most recent therapy. The Company also plans to submit an MAA to the EMA in early 2019 with a request for conditional approval in the same indication.

- **Reported Positive Top-line Data from the Phase 2b STORM Study in Patients with Penta-Refractory Myeloma.** Karyopharm reported positive top-line results from Part 2 of the Phase 2b STORM study evaluating selinexor plus low dose dexamethasone (Sd) in patients with penta-refractory multiple myeloma. The STORM study's primary endpoint of overall response rate (ORR) was 25.4%, which included two stringent complete responses (sCRs) and 29 partial or very good partial responses. The two sCRs were negative for minimal residual disease, one at $1{:}10^{-6}$ and one at $1{:}10^{-4}$, which is particularly significant in this penta-refractory population. The median duration of response, a key secondary objective, was 4.4 months. Across the relevant patient population, side effects of oral selinexor were generally predictable and often managed with dose adjustments or supportive care. 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.

- **Presented Updated Data from the Phase 1b/2 STOMP Study at the European Hematology Association (EHA) 2018 Annual Meeting.** At EHA 2018, Karyopharm presented three posters with updated data from STOMP evaluating selinexor and dexamethasone in combination with standard approved therapies, Velcade (SVd), Pomalyst (SPd) or Darzalex (SDd), in patients with previously treated multiple myeloma. The SVd arm demonstrated progression-free survival (PFS) of 17.8 months, an ORR of 83% in the same patient population eligible for the pivotal Phase 3 BOSTON study. The SDd arm demonstrated an ORR of 82% in patients with heavily pretreated Darzalex-naïve disease. The all oral SPd arm demonstrated an ORR of 55% in patients with Pomalyst-naïve and Revlimid-relapsed or -refractory disease,

with a PFS of 11.6 months. Adverse events across all three arms were consistent with those reported previously with selinexor and the combination therapies, with no new safety signals specific to the combinations identified. These results suggest that selinexor can be combined with other anti-myeloma agents and induce durable responses in patients with previously treated MM.

- **Initiated New STOMP Arm Evaluating All Oral Regimen of Selinexor, Revlimid and Dexamethasone (SRd) in Patients with Newly Diagnosed Myeloma.** Based on the positive STOMP results reported to date evaluating SRd in patients with relapsed myeloma, the Company initiated a new, all oral STOMP arm to investigate the combination of SRd in the front-line setting. Given the observed synergistic activity of selinexor with standard approved multiple myeloma therapies, Karyopharm believes oral selinexor has the potential to be a future backbone therapy in multiple myeloma.
- **Pivotal Phase 3 BOSTON Study in Progress.** Karyopharm's pivotal, randomized Phase 3 BOSTON study is underway and enrolling patients in 14 countries. BOSTON is evaluating 100mg of selinexor dosed once weekly in combination with the proteasome inhibitor Velcade (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. The primary endpoints of the study are PFS and ORR. Data from the BOSTON study, if positive, would be used to support regulatory submissions to the FDA and EMA requesting full approvals for use of selinexor in second line multiple myeloma, following the Company's requests for accelerated and conditional approvals, respectively, using data from the Phase 2b STORM study. 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 who are not eligible for stem cell transplantation. 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 to five lines of prior therapy. Karyopharm plans to report top-line results by the end of 2018. Assuming the results from the SADAL study are positive, Karyopharm plans to submit an NDA to the FDA with a request for accelerated approval, and an MAA to the EMA with a request for conditional approval, for oral selinexor in this relapsed/refractory DLBCL patient population.

*Selinexor in Solid Tumors*

- **Presented Data from the Phase 2 Portion of the Phase 2/3 SEAL Study in Liposarcoma at the American Society of Clinical Oncology (ASCO) 2018 Annual Meeting.** At ASCO 2018, Karyopharm presented positive results from the successful 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 primary endpoint of PFS, oral selinexor showed superiority over placebo, achieving a median PFS of 5.5 months, compared to 2.7 months for placebo with a hazard ratio (HR) of 0.67, representing a 33% reduction in the risk of progression or death. Across the relevant patient population, side effects of oral selinexor were generally predictable and often managed with dose adjustments or supportive care, with the most frequent events being nausea, fatigue, anorexia and weight loss, with low levels of Grade 3/4 cytopenias, and no new or unexpected safety signals identified. The Phase 3 portion of the SEAL study is underway and, assuming a positive outcome on the primary end point of PFS, the Company intends to use the data from the SEAL study to support an NDA and an MAA submission requesting full approval 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. In the U.S., endometrial cancer is the most common gynecological cancer with approximately 58,000 cases expected to be diagnosed and an estimated 10,000 women expected to die from this cancer in 2018[1], revealing a meaningful patient population in need of novel therapies.

*Corporate Updates*

- **Anand Varadan Appointed Chief Commercial Officer.** Karyopharm announced the appointment of Anand Varadan as Executive Vice President, Chief Commercial Officer. Mr. Varadan brings over 25 years of commercial operations and strategy experience with a proven track record of building and leading commercial and cross-functional teams and successfully launching and marketing new therapeutics at publicly-traded, healthcare-focused companies. Mr. Varadan leads the Company's commercial strategy and operations, including for the launch of selinexor.

- **Ian Karp Appointed Vice President, Investor and Public Relations.** The Company also appointed Ian Karp as Vice

President, Investor and Public Relations. Mr. Karp brings over 20 years of investor relations, corporate development, and commercial experience and has successfully led global teams in achieving key corporate communications objectives, including in the areas of oncology and orphan diseases. Mr. Karp leads all of the Company's corporate communications activities, including corporate visibility, financial communications, and media and investor relations.

- **Exclusive License Agreement Executed with Antengene to Develop and Commercialize Selinexor, Eltanexor, Verdinexor and KPT-9274 in China and Other Regions in Asia.** The agreement includes the development of selinexor and eltanexor for the diagnosis, treatment and/or prevention of all human oncology indications in China and Macau. The agreement also includes the development and commercialization of KPT-9274 in all human oncology indications and verdinexor in human non-oncology indications in mainland China, Macau, Taiwan, Hong Kong, South Korea, and the ASEAN countries. The transaction carries a total deal value of up to $162 million, plus royalties.

**Second Quarter Ended June 30, 2018 Financial Results**

Cash, cash equivalents and investments as of June 30, 2018, including restricted cash, totaled $250.5 million, compared to $176.4 million as of December 31, 2017.

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, resulting in net proceeds of approximately $145.7 million after deducting the underwriting discounts and commissions and other offering expenses.

For the quarter ended June 30, 2018, Karyopharm recognized $19.9 million in revenue, compared to a small amount of grant revenue for the three months ended June 30, 2017. The increase in revenue was primarily the result of recognizing $19.7 million of revenue related to fulfilling an obligation under our license agreement with Ono Pharmaceutical Co., LTD. The cash related to this revenue was received as part of the upfront payment received from Ono in October 2017.

For the quarter ended June 30, 2018, research and development expense was $44.7 million compared to $23.1 million for the quarter ended June 30, 2017. For the quarter ended June 30, 2018, general and administrative expense was $9.5 million compared to $6.6 million for the quarter ended June 30, 2017.

Karyopharm reported a net loss of $33.7 million, or $0.60 per share, for the quarter ended June 30, 2018, compared to a net loss of $29.4 million, or $0.64 per share, for the quarter ended June 30, 2017. Net loss includes stock-based compensation expense of $4.4 million and $5.1 million for the quarters ended June 30, 2018 and June 30, 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 for at least the next twelve months. These plans include the continued clinical development of selinexor in the Company's lead indications with a focus on preparing the commercial infrastructure and hiring a sales force for the potential launch of selinexor in the U.S. Additional key activities 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 overall response rate (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 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 ongoing pivotal, Phase 3 BOSTON study, and has reminded the Company that accelerated approval requires patients to have exhausted all available approved therapies. FDA's Fast Track designation is available to therapeutics treating an unmet medical need in a serious condition; the Company has received Fast Track designation from the FDA specifically for the population treated in the STORM trial. In light of this recognition that the STORM patient population represents an unmet medical need and the positive top-line data reported in April 2018, 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, Tuesday, August 7, 2018, at 8:30 a.m. Eastern Time, to discuss the second 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 3084449. 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 2,600 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. For the STORM study's primary objective, oral selinexor achieved a 25.4% overall

response rate, which included two stringent complete responses, both of which were negative for minimal residual disease, and 29 partial or very good partial responses. The median duration of response, a key secondary objective, was 4.4 months, and patients with any response had a significantly prolonged overall survival as compared with patients who did not respond. Selinexor has been granted Orphan Drug Designation in multiple myeloma and Fast Track designation for the patient population evaluated in the STORM study. Karyopharm has completed a rolling submission for a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA), 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), as a potential backbone therapy in combination with approved therapies (STOMP), 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 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, 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 plans for commercialization, 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 Quarterly Report on Form 10-Q for the quarter ended March 31, 2018, which was filed with the Securities and Exchange Commission (SEC) on May 10, 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.



**References**

[1] American Cancer Society. https://www.cancer.org/cancer/endometrial-cancer/about/key-statistics.html

**Contacts:**

**Investors:**
Karyopharm Therapeutics Inc.
Ian Karp
Vice President, Investor and Public Relations
857-297-2241 | ikarp@karyopharm.com

**Media:**
Argot Partners
David Rosen
212-600-1902 | david.rosen@argotpartners.com

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

| | June 30, 2018 | December 31, 2017 |
|---|---|---|
| **Assets** | | |
| Current assets: | | |
| Cash and cash equivalents | $ 118,966 | $ 68,997 |
| Short-term investments | 122,155 | 77,472 |
| Prepaid expenses and other current assets | 3,438 | 1,754 |
| Restricted cash | — | 200 |
| Total current assets | 244,559 | 148,423 |
| Property and equipment, net | 2,611 | 2,185 |
| Long-term investments | 8,781 | 29,396 |
| Restricted cash | 638 | 290 |
| Total assets | $ 256,589 | $ 180,294 |
| **Liabilities and stockholders' equity** | | |
| Current liabilities: | | |
| Accounts payable | $ 3,794 | $ 5,665 |
| Accrued expenses | 22,969 | 21,445 |
| Deferred revenue | 9,363 | 21,921 |
| Deferred rent | 189 | 303 |
| Other current liabilities | 229 | 133 |
| Total current liabilities | 36,544 | 49,467 |
| Deferred revenue, net of current portion | 4,532 | — |
| Deferred rent, net of current portion | 2,041 | 1,363 |
| Total liabilities | 43,117 | 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; 60,501,260 and 49,533,150 shares issued and outstanding at June 30, 2018 and December 31, 2017, respectively | 6 | 5 |
| Additional paid-in capital | 781,180 | 625,017 |
| Accumulated other comprehensive loss | (260) | (217) |
| Accumulated deficit | (567,454) | (495,341) |
| Total stockholders' equity | 213,472 | 129,464 |
| Total liabilities and stockholders' equity | $ 256,589 | $ 180,294 |

**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, | |
|---|---|---|---|---|
| | 2018 | 2017 | 2018 | 2017 |
| License and other revenue | $ 19,891 | $ 3 | $ 29,891 | $ 71 |
| Operating expenses: | | | | |
| Research and development | 44,734 | 23,120 | 86,055 | 47,203 |
| General and administrative | 9,489 | 6,635 | 17,110 | 12,899 |

| | | | | |
|---|---|---|---|---|
| Total operating expenses | 54,223 | 29,755 | 103,165 | 60,102 |
| Loss from operations | (34,332) | (29,752) | (73,274) | (60,031) |
| Other income (expense): | | | | |
| Interest income | 653 | 412 | 1,162 | 812 |
| Other income (expense) | 7 | (29) | (7) | (44) |
| Total other income, net | 660 | 383 | 1,155 | 768 |
| Loss before income taxes | (33,672) | (29,369) | (72,119) | (59,263) |
| Income tax benefit (provision) | 17 | (18) | 5 | (41) |
| Net loss | $ (33,655) | $ (29,387) | $ (72,114) | $ (59,304) |
| Net loss per share—basic and diluted | $ (0.60) | $ (0.64) | $ (1.36) | $ (1.35) |
| Weighted-average number of common shares outstanding used in net loss per share—basic and diluted | 56,089,159 | 45,831,239 | 52,862,194 | 43,873,892 |

Source: Karyopharm Therapeutics Inc.