

## Karyopharm Reports First Quarter 2020 Financial Results and Highlights Recent Company Progress

*-- Pivotal Phase 3 BOSTON Study Meets Primary Endpoint with Significant Increase in Progression-Free Survival; Oral Presentation at ASCO 2020 Virtual Scientific Program and sNDA To Be Submitted by End of May 2020 --*

*-- First Quarter 2020 XPOVIO Net Product Sales of \$16.1 Million and Total Revenues of \$18.1 Million --*

*-- To Receive \$12.0 Million from Antengene for Expanded Territory --*

*-- sNDA for Selinexor in DLBCL Accepted by FDA and Granted Priority Review; Assigned PDUFA Target Action Date of June 23, 2020 --*

*-- Initiated Randomized Study Evaluating Low Dose Selinexor in Patients with Severe COVID-19 --*

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

**NEWTON, Mass.** – May 5, 2020 – Karyopharm Therapeutics Inc. (Nasdaq:KPTI), an innovation-driven pharmaceutical company, today reported financial results for the quarter ended March 31, 2020. In addition, Karyopharm highlighted select corporate milestones, including details regarding the ongoing U.S. commercialization of XPOVIO® (selinexor), and provided an overview of its key clinical development programs.

“I am extremely proud of the significant progress we have made thus far in 2020. Following our recent announcement of the positive top-line results from the pivotal Phase 3 BOSTON study, we are actively preparing to share the dataset at the American Society of Clinical Oncology 2020 (ASCO20) Virtual Scientific Program and expect to submit a supplemental New Drug Application (sNDA) to the U.S. Food and Drug Administration (FDA) by the end of May 2020, requesting an expansion of the currently approved indication for XPOVIO to include second line treatment for patients with relapsed or refractory multiple myeloma,” said Michael G. Kauffman, MD, PhD, Chief Executive Officer of Karyopharm. “Additionally, despite industry-wide challenges created by the current COVID-19 pandemic, we remain pleased by the ongoing commercialization of XPOVIO in the U.S., as well as our ability to significantly strengthen our balance sheet in the first quarter. Finally, I want to assure our many stakeholders that the entire Karyopharm team has risen to the challenge of navigating the Company through these difficult times and we are working day and night to provide critical medicines to the patients and physicians who need them most. In addition to the applications in oncology, we remain highly encouraged by the potential antiviral and anti-inflammatory activity of XPO1 inhibition with selinexor and look forward to working, as quickly as possible, with the medical community of regulators, treating physicians and patients on advancing our new clinical study of low dose selinexor to treat patients with severe COVID-19.”

### First Quarter 2020 and Recent Highlights

#### *XPOVIO in Multiple Myeloma*

- **Pivotal Phase 3 BOSTON Study Met Primary Endpoint.** In early March 2020, Karyopharm announced the top-line results from the BOSTON study, including that BOSTON met its primary endpoint of a statistically significant increase in progression-free survival (PFS). 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. The median PFS in the SVd arm was 13.93 months compared to 9.46 months in the Vd arm, representing a 4.47 month (47%) increase in median PFS (hazard ratio=0.70; p=0.0066). There were no new safety signals on the SVd arm and there was no imbalance in deaths between the two arms in the study. The clinical data will be disclosed in an oral presentation at the ASCO 2020 Virtual Scientific Program taking place May 29-31, 2020. Karyopharm expects to submit an sNDA to the FDA by the end of May 2020 requesting expansion of the currently approved indication for XPOVIO to include second line treatment for patients with relapsed or refractory multiple myeloma. A regulatory submission to the European Medicines Agency (EMA) is also planned.

- **XPOVIO U.S. Commercialization.** Oral XPOVIO became commercially available to patients in the U.S. in July 2019 and generated net product sales of \$16.1 million in the first quarter of 2020. As of March 31, 2020, more than 2,200 XPOVIO prescriptions have been filled since launch, and more than 150 new physician prescribing accounts were added in the first quarter. Prescription refills for XPOVIO among existing patients remained strong in the quarter. Based on data from specialty pharmacies, approximately 60% of eligible patients have received a refill for their second prescription since launch, and the per patient average number of prescriptions continues to increase each quarter. Additionally, for those patients responding well to therapy, we continue to see an increasing and meaningful number of patients who have now been on therapy for five months or longer, further supporting the potential for longer-term use of XPOVIO therapy, as appropriate. We did experience a decrease in net product sales in the first quarter of 2020 compared to the fourth quarter of 2019, largely driven by minimal channel inventory build in the first quarter. Finally, there were fewer new patient XPOVIO starts than expected in the quarter, primarily due to the diversion of physician and other healthcare resources to manage the ongoing COVID-19 pandemic and the significant reduction in Karyopharm's field force in-person activities as a result of shelter-in-place orders across the U.S. However, we were encouraged to see an acceleration in demand for XPOVIO in April 2020.
- **Decision from EMA for Marketing Authorization Application (MAA) Expected in 2020.** In January 2019, Karyopharm submitted an 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. In January 2020, Karyopharm was granted a three-month extension from the EMA's Committee for Medicinal Products for Human Use (CHMP) to provide additional time to respond to the outstanding questions from the MAA, primarily re-monitoring certain clinical data. Due to the COVID-19 pandemic and the resulting disruption at many clinical sites, re-monitoring activities requested by CHMP remain ongoing. The Company does not anticipate any significant changes to the data sets supporting the MAA filing from the ongoing re-monitoring activities. The Company now expects to be able to respond to CHMP in mid-2020 and to receive a decision on the selinexor MAA by late-2020.

#### *Selinexor in Diffuse Large B-Cell Lymphoma (DLBCL)*

- **FDA Accepts sNDA and Grants Priority Review.** The FDA accepted for filing with Priority Review Karyopharm's sNDA seeking accelerated approval for selinexor for the treatment of adult patients with relapsed or refractory DLBCL, not otherwise specified, who have received at least two prior therapies. The FDA assigned an action date of June 23, 2020 under the Prescription Drug User Fee Act (PDUFA). The Company also expects to submit an MAA to the EMA in 2021 requesting conditional approval for selinexor in the same indication. In addition to Orphan Drug Designation, selinexor was granted Fast Track designation for this indication by the FDA in 2018.

*Low Dose Selinexor in COVID-19*

- **First Patient Dosed in Randomized Study.** Karyopharm recently announced the initiation of a new study to evaluate low dose oral selinexor in hospitalized patients with severe COVID-19. SINE compounds, including both selinexor and its close relative verdinexor, have been identified as having the potential to interfere with key host protein interactions with influenza, RSV and other viruses, including SARS-CoV-2, the virus that causes COVID-19. Furthermore, XPO1 (also called CRM1) was identified as one of the host proteins with the highest number of functional connections with SARS-CoV proteins. Recent preclinical experiments have further demonstrated selinexor's ability to inhibit the viral propagation of the SARS-CoV-2 virus in monkey Vero cells, which are commonly used to study human viral infections. Finally, SINE compounds, including selinexor, have demonstrated potent anti-inflammatory activity through the inhibition of Nuclear Factor  $\kappa$ B (NF- $\kappa$ B), leading to reductions in cytokines such as IL6, IL1, IFN $\gamma$  and others in a variety of models. Since high levels of these cytokines are found in patients with COVID-19 and the most severe disease, reductions in these cytokines may be particularly beneficial to hospitalized patients with COVID-19. Patient enrollment and dosing has begun in this randomized, placebo-controlled, Phase 2 study (XPORT-CoV-1001 / NCT04349098), which is expected to enroll approximately 230 patients at clinical sites in the U.S., Europe, and Israel.

*Corporate and Financial Updates*

- **Expanded Territory Rights with Antengene Corporation and Reacquisition of Rights from Ono Pharmaceutical Co., Ltd.** Karyopharm's license agreement with Antengene, our current partner in China, was amended to provide Antengene with the exclusive right to develop and commercialize selinexor and eltanexor in all human oncology indications in Australia, New Zealand, South Korea, Taiwan, Hong Kong and the ASEAN countries. The amended agreement also includes the development and commercialization of KPT-9274 in all human oncology indications and verdinexor in human non-oncology indications in Australia and New Zealand. Under the terms of the amended agreement, Karyopharm will receive a one-time upfront payment of \$12.0 million from Antengene, expected in the second quarter of 2020. Karyopharm is eligible to receive additional payments if certain future prespecified regulatory and commercial milestones are achieved by Antengene. Karyopharm is also eligible to receive tiered double-digit royalties based on future net sales of selinexor and eltanexor, and tiered single-to double-digit royalties based on future net sales of verdinexor and KPT-9274 in the expanded territory.

Certain countries in the expanded territory with Antengene became available following Karyopharm's reacquisition of the exclusive development and commercial rights from Ono which were transferred to Karyopharm in April 2020 at no cost to Karyopharm. Karyopharm has chosen to retain the rights to selinexor and eltanexor in Japan while granting Antengene the exclusive rights to develop and commercialize selinexor and eltanexor in South Korea, Taiwan, Hong Kong and the ASEAN countries.

Outside of the territories licensed by Antengene, and the market for selinexor in Israel, Karyopharm maintains complete development and commercial rights to selinexor and eltanexor throughout the world, including in the U.S., Canada, Europe, Japan, and Latin America.

- **Strengthened the Balance Sheet with a Public Equity Offering.** On March 6, 2020, Karyopharm completed an underwritten public offering of 7,187,500 shares of its common stock at a price to the public of \$24.00 per

share. The net proceeds of \$161.8 million from the offering will be used to support key research and development and commercial initiatives, including potential future product launches.

- **Collaboration Agreement with Curadev Pharma Pvt Ltd (Curadev).** Karyopharm and Curadev, a privately-owned biotechnology company, have entered into a strategic collaboration to identify and co-develop novel small molecules against various biological targets for the treatment of cancer and other major diseases. Under the terms of the agreement, Karyopharm and Curadev have agreed to identify and develop small molecules against up to two targets. Curadev will conduct exploratory research, drug discovery and development for designated programs up to the conclusion of preclinical proof of concept studies, after which Karyopharm will have an option to an exclusive license to develop and commercialize each target on a global basis. Karyopharm and Curadev will co-fund and jointly oversee development up to the option exercise period.
- **Key Appointments to the Executive Leadership Team and Board of Directors.** During the first quarter of 2020, Karyopharm appointed John Demaree as Chief Commercial Officer and its Board of Directors elected Richard Paulson as a director. Mr. Demaree brings more than 20 years of oncology experience, building commercial capabilities and leading multiple successful product launches. Mr. Paulson currently serves as Executive Vice President and Chief Executive Officer of Ipsen North America and brings over 25 years of global biopharmaceutical industry experience, including launching best-in-class oncology medicines.

### First Quarter 2020 Financial Results

**Net product revenue:** Net product revenue for the first quarter of 2020 was \$16.1 million. Karyopharm commenced sales of XPOVIO in the U.S. during the third quarter of 2019 and therefore did not have net product revenue during the first quarter of 2019.

**License and other revenue:** License and other revenue for the first quarter of 2020 was \$2.1 million, compared to \$0.2 million for the first quarter of 2019. The increase was driven in part by the recognition of \$1.1 million pursuant to our license agreement with Antengene.

**Cost of sales:** Cost of sales were \$0.8 million for the first quarter of 2020. Cost of sales reflects the costs of XPOVIO units sold and third-party royalties on net product revenue.

**Research and development expenses (R&D):** R&D expenses for the first quarter of 2020 were \$34.0 million, compared to \$38.0 million for the first quarter of 2019.

**Selling, general and administrative expenses (SG&A):** For the first quarter of 2020, SG&A expenses were \$30.7 million, compared to \$27.1 million for the first quarter of 2019. The increase in SG&A expenses compared to the prior year was due primarily to activities to support the U.S. commercialization of XPOVIO and in preparation for the potential launch of additional indications in 2020.

**Interest expense:** Interest expense for the first quarter of 2020 was \$6.5 million, compared to \$3.0 million for the first quarter 2019. The increase in interest expense is attributable to the imputed interest on the deferred royalty obligation Karyopharm has with HealthCare Royalty Partners.

**Net loss:** Karyopharm reported a net loss of \$52.9 million, or \$0.78 per share, for the first quarter of 2020, compared to a net loss of \$66.2 million, or \$1.09 per share, for the first quarter of 2019. Net loss includes non-

cash stock-based compensation expense of \$5.2 million and \$3.9 million for the 2020 and 2019 quarters, respectively.

**Cash position:** Cash, cash equivalents, restricted cash and investments as of March 31, 2020 totaled \$385.2 million, compared to \$265.8 million as of December 31, 2019.

### 2020 Financial Outlook

Karyopharm expects XPOVIO net product sales to be slightly higher in the second quarter of 2020 as compared to the first quarter of 2020. In addition, total revenues are expected to be higher due to an increase in collaboration revenue from the expanded territory agreement with Antengene. The Company will not be issuing XPOVIO revenue guidance for the full year 2020 as it continues to monitor the ongoing commercial impact from the COVID-19 pandemic as well as the timing of key expected regulatory actions in 2020. These regulatory events include the potential approval of XPOVIO for patients with relapsed or refractory DLBCL as well as Karyopharm's planned sNDA submission, and subsequent FDA-review period, requesting expansion of the approved indication for XPOVIO to include second line treatment for patients with relapsed or refractory multiple myeloma.

Based on its current operating plans, including the reduction of some R&D costs as a result of trial delays due to the ongoing COVID-19 pandemic, Karyopharm expects its non-GAAP R&D and SG&A expenses, which excludes stock-based compensation expense, for the full year 2020 to be at the lower end of the previously projected range of \$240 million to \$260 million. This estimate includes the additional costs associated with our new selinexor clinical trial in patients with severe COVID-19. Karyopharm has not reconciled the full year 2020 outlook for non-GAAP R&D and SG&A expenses to full year 2020 outlook for GAAP R&D and SG&A expenses 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 full year 2020 outlook for non-GAAP R&D and SG&A expenses.

The Company expects that its existing cash, cash equivalents and investments, and the revenue it expects to generate from XPOVIO product sales, will be sufficient to fund its planned operations into the middle of 2022.

### Non-GAAP Financial Information

Karyopharm uses a non-GAAP financial measure, including R&D and SG&A expenses, to provide operating expense guidance. Non-GAAP R&D and SG&A expenses exclude stock-based compensation expense. 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 R&D and SG&A expenses and should not be considered a measure of Karyopharm's liquidity. Instead, non-GAAP R&D and SG&A expenses should only be used to supplement an understanding of Karyopharm's operating results as reported under GAAP.

### Conference Call Information

Karyopharm will host a conference call today, Tuesday, May 5, 2020, at 8:30 a.m. Eastern Time, to discuss the first quarter 2020 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 9936655. 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**

### **Thrombocytopenia**

XPOVIO can cause thrombocytopenia, leading to potentially fatal hemorrhage. Thrombocytopenia was reported as an adverse reaction in 74% of patients, and severe (Grade 3-4) thrombocytopenia occurred in 61% of patients treated with XPOVIO. The median time to onset of the first event was 22 days. Bleeding occurred in 23% of patients with thrombocytopenia, clinically significant bleeding occurred in 5% of patients with thrombocytopenia and fatal hemorrhage occurred in <1% of patients.

Monitor platelet counts at baseline, during treatment, and as clinically indicated. Monitor more frequently during the first two months of treatment. Institute platelet transfusion and/or other treatments as clinically indicated. Monitor patients for signs and symptoms of bleeding and evaluate promptly. Interrupt and/or reduce dose, or permanently discontinue based on severity of adverse reaction.

### **Neutropenia**

XPOVIO can cause neutropenia, potentially increasing the risk of infection. Neutropenia was reported as an adverse reaction in 34% of patients, and severe (Grade 3-4) neutropenia occurred in 21% of patients treated with XPOVIO. The median time to onset of the first event was 25 days. Febrile neutropenia was reported in 3% of patients.

Obtain neutrophil counts at baseline, during treatment, and as clinically indicated. Monitor more frequently during the first two months of treatment. Monitor patients for signs and symptoms of concomitant infection and evaluate promptly. Consider supportive measures including antimicrobials for signs of infection and use of growth factors (e.g., G-CSF). Interrupt and/or reduce dose, or permanently discontinue based on severity of adverse reaction.

### **Gastrointestinal Toxicity**

Gastrointestinal toxicities occurred in patients treated with XPOVIO.

#### **Nausea/Vomiting**

Nausea was reported as an adverse reaction in 72% of patients, and Grade 3 nausea occurred in 9% of patients treated with XPOVIO. The median time to onset of the first nausea event was 3 days.

Vomiting was reported in 41% of patients, and Grade 3 vomiting occurred in 4% of patients treated with XPOVIO. The median time to onset of the first vomiting event was 5 days.

Provide prophylactic 5-HT<sub>3</sub> antagonists and/or other anti-nausea agents, prior to and during treatment with XPOVIO. Manage nausea/vomiting by dose interruption, reduction, and/or discontinuation. Administer intravenous fluids and replace electrolytes to prevent dehydration in patients at risk. Use additional anti-nausea medications as clinically indicated.

**Diarrhea**

Diarrhea was reported as an adverse reaction in 44% of patients, and Grade 3 diarrhea occurred in 6% of patients treated with XPOVIO. The median time to onset of diarrhea was 15 days.

Manage diarrhea by dose modifications and/or standard anti-diarrheal agents; administer intravenous fluids to prevent dehydration in patients at risk.

**Anorexia/Weight Loss**

Anorexia was reported as an adverse reaction in 53% of patients, and Grade 3 anorexia occurred in 5% of patients treated with XPOVIO. The median time to onset of anorexia was 8 days.

Weight loss was reported as an adverse reaction in 47% of patients, and Grade 3 weight loss occurred in 1% of patients treated with XPOVIO. The median time to onset of weight loss was 15 days.

Monitor patient weight at baseline, during treatment, and as clinically indicated. Monitor more frequently during the first two months of treatment. Manage anorexia and weight loss with dose modifications, appetite stimulants, and nutritional support.

**Hyponatremia**

XPOVIO can cause hyponatremia; 39% of patients treated with XPOVIO experienced hyponatremia, 22% of patients experienced Grade 3 or 4 hyponatremia. The median time to onset of the first event was 8 days.

Monitor sodium level at baseline, during treatment, and as clinically indicated. Monitor more frequently during the first two months of treatment. Correct sodium levels for concurrent hyperglycemia (serum glucose >150 mg/dL) and high serum paraprotein levels. Treat hyponatremia per clinical guidelines (intravenous saline and/or salt tablets), including dietary review. Interrupt and/or reduce dose, or permanently discontinue based on severity of adverse reaction.

**Infections**

In patients receiving XPOVIO, 52% of patients experienced any grade of infection. Upper respiratory tract infection of any grade occurred in 21%, pneumonia in 13%, and sepsis in 6% of patients. Grade  $\geq 3$  infections were reported in 25% of patients, and deaths resulting from an infection occurred in 4% of patients. The most commonly reported Grade  $\geq 3$  infections were pneumonia in 9% of patients, followed by sepsis in 6%. The median time to onset was 54 days for pneumonia and 42 days for sepsis. Most infections were not associated with neutropenia and were caused by non-opportunistic organisms.

**Neurological Toxicity**

Neurological toxicities occurred in patients treated with XPOVIO.

Neurological adverse reactions including dizziness, syncope, depressed level of consciousness, and mental status changes (including delirium and confusional state) occurred in 30% of patients, and severe events (Grade 3-4) occurred in 9% of patients treated with XPOVIO. Median time to the first event was 15 days.

Optimize hydration status, hemoglobin level, and concomitant medications to avoid exacerbating dizziness or mental status changes.

### **Embryo-Fetal Toxicity**

Based on data from animal studies and its mechanism of action, XPOVIO can cause fetal harm when administered to a pregnant woman. Selinexor administration to pregnant animals during organogenesis resulted in structural abnormalities and alterations to growth at exposures below those occurring clinically at the recommended dose.

Advise pregnant women of the potential risk to a fetus. Advise females of reproductive potential and males with a female partner of reproductive potential to use effective contraception during treatment with XPOVIO and for 1 week after the last dose.

### **ADVERSE REACTIONS**

The most common adverse reactions (incidence  $\geq 20\%$ ) 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%.

**Please see XPOVIO Full Prescribing Information available at [www.XPOVIO.com](http://www.XPOVIO.com).**

### **About Karyopharm Therapeutics**

Karyopharm Therapeutics Inc. (Nasdaq: KPTI) is an innovation-driven 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 Selective Inhibitor of Nuclear Export (SINE) compounds function by binding with and inhibiting the nuclear export protein XPO1 (or CRM1). Karyopharm's lead compound, XPOVIO<sup>®</sup> (selinexor), received accelerated approval from the U.S. Food and Drug Administration (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. A supplemental New Drug Application was accepted by the FDA seeking accelerated approval for selinexor as a new treatment for adult patients with relapsed or refractory diffuse large B-cell lymphoma (DLBCL). 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](http://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 and plans relating to selinexor as a potential treatment for patients with COVID-19; the design and execution of

Karyopharm's clinical trials to study this potential application of selinexor, including the dosing regimen; the potential anti-viral and anti-inflammatory properties of selinexor; XPOVIO for the treatment of patients with heavily pretreated multiple myeloma or relapsed or refractory diffuse large B-cell lymphoma; commercialization of XPOVIO or any of its drug candidates and the commercial performance of XPOVIO; submissions to, and the review and potential approval of selinexor by, regulatory authorities, including the anticipated availability of data to support such submissions, timing of such submissions and actions by regulatory authorities and the potential availability of accelerated approval pathways; the therapeutic potential of and potential clinical development plans for Karyopharm's drug candidates, especially selinexor; 2020 financial expectations, including forecasted non-GAAP R&D and SG&A expenses; and expectations of the sufficiency of Karyopharm's existing cash and investments. 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 confirmatory approval in the U.S. or EU based on the BOSTON study in patients with relapsed or refractory multiple myeloma, or accelerated approval in the U.S. for patients with relapsed or refractory DLBCL as a result of data from the SADAL study, 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 the development or commercialization of 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 risk that the COVID-19 pandemic could disrupt Karyopharm's business more severely than it currently anticipates, including by reducing sales of XPOVIO, interrupting or delaying research and development efforts, impacting the ability to procure sufficient supply for the development and commercialization of selinexor or other product candidates, delaying ongoing or planned clinical trials, impeding the execution of business plans, planned regulatory milestones and timelines, or inconveniencing patients; the adoption of selinexor for treatment of COVID-19, if approved, in the commercial marketplace, the timing and costs involved in commercializing selinexor for such indication; the adoption of XPOVIO in the commercial marketplace, 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; the ability of Karyopharm or its third party collaborators or successors in interest to fully perform their respective obligations under the applicable agreement and the potential future financial implications of such agreement; 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, 2019, which was filed with the Securities and Exchange Commission (SEC) on February 26, 2020, 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.



## Targeting Disease at the Nuclear Pore

### **Contacts:**

#### *Investors:*

Karyopharm Therapeutics Inc.

Ian Karp, Vice President, Investor and Public Relations

857-297-2241 | [ikarp@karyopharm.com](mailto:ikarp@karyopharm.com)

#### *Media:*

FTI Consulting

Simona Kormanikova or Robert Stanislaro

212-850-5600 | [Simona.Kormanikova@fticonsulting.com](mailto:Simona.Kormanikova@fticonsulting.com) or [robert.stanislaro@fticonsulting.com](mailto:robert.stanislaro@fticonsulting.com)

**KARYOPHARM THERAPEUTICS INC.**  
**CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS**  
**(unaudited)**  
**(in thousands, except per share amounts)**

	<b>Three Months Ended, March 31,</b>	
	<b>2020</b>	<b>2019</b>
Revenues:		
Product revenue, net	\$ 16,061	\$ —
License and other revenue	2,077	155
Total revenues	<u>18,138</u>	<u>155</u>
Operating expenses:		
Cost of sales	819	—
Research and development	33,997	37,974
Selling, general and administrative	30,678	27,103
Total operating expenses	<u>65,494</u>	<u>65,077</u>
Loss from operations	(47,356)	(64,922)
Other income (expense):		
Interest income	975	1,771
Interest expense	(6,509)	(2,998)
Other income (expense), net	25	(2)
Total other expense, net	<u>(5,509)</u>	<u>(1,229)</u>
Loss before income taxes	(52,865)	(66,151)
Income tax provision	(66)	(10)
Net loss	<u>\$ (52,931)</u>	<u>\$ (66,161)</u>
Net loss per share—basic and diluted	<u>\$ (0.78)</u>	<u>\$ (1.09)</u>
Weighted-average number of common shares outstanding used in net loss per share—basic and diluted	<u>67,627</u>	<u>60,856</u>



## Targeting Disease at the Nuclear Pore

**Karyopharm Therapeutics Inc.**  
**Condensed Consolidated Balance Sheets**  
**(in thousands)**  
**(unaudited)**

	<u>March 31,</u> <u>2020</u>	<u>December 31,</u> <u>2019</u>
<b>ASSETS</b>		
Cash, cash equivalents and investments	\$ 383,504	\$ 263,972
Restricted cash	1,701	1,831
Accounts receivable	9,281	7,862
Property and equipment, net	2,814	3,046
Other assets	18,841	18,252
Total assets	<u>\$ 416,141</u>	<u>\$ 294,963</u>
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Deferred revenue	3,479	4,533
Convertible senior notes	111,769	109,857
Deferred royalty obligation	73,588	73,588
Other liabilities	61,158	57,211
Total liabilities	<u>249,994</u>	<u>245,189</u>
Total stockholders' equity	<u>166,147</u>	<u>49,774</u>
Total liabilities and stockholders' equity; 73,095 and 65,370 shares issued and outstanding at March 31, 2020 and December 31, 2019, respectively	<u>\$ 416,141</u>	<u>\$ 294,963</u>