

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

**-- Phase 3 SEAL Study Meets Primary Endpoint with Significant Increase in Progression-Free Survival in Patients with Unresectable Dedifferentiated Liposarcoma --**

**-- Third Quarter 2020 XPOVIO Net Product Sales of \$21.3 Million; Quarterly Sales Continue Strong Momentum, Increasing Approximately 15% Compared to the Second Quarter of 2020 --**

**-- XPOVIO Supplemental New Drug Application Seeking Approval for Patients with Multiple Myeloma After At Least One Prior Line of Therapy Assigned a Target PDUFA Action Date of March 19, 2021 --**

**-- Conference Call Scheduled for Today at 4:30 p.m. ET --**

NEWTON, Mass., Nov. 2, 2020 [/PRNewswire/](#) -- Karyopharm Therapeutics Inc. (Nasdaq: KPTI), a commercial-stage pharmaceutical company pioneering novel cancer therapies, today reported financial results for the quarter ended September 30, 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.

"We are delighted to share the significant top-line results from the Phase 3 portion of the SEAL study, the first, late-stage clinical data for XPOVIO in a solid tumor indication," said Sharon Shacham, PhD, MBA, President and Chief Scientific Officer of Karyopharm. "The top-line results from the SEAL study are particularly encouraging as advanced dedifferentiated liposarcoma represents a very difficult to treat cancer with no established standard of care and limited treatment options available to patients. XPOVIO may be particularly promising as it represents the first oral therapy to show activity in patients with previously treated liposarcoma. We look forward to presenting the detailed results at the upcoming Connective Tissue Oncology Society (CTOS) Annual Meeting and plan to submit a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) in the first quarter of 2021 requesting approval of XPOVIO to treat the patient population studied in SEAL. If approved, XPOVIO would represent the first oral, non-chemotherapy agent available for patients with dedifferentiated liposarcoma. The encouraging data from the SEAL study also provide additional rationale for advancing the clinical development of XPOVIO in other solid tumor indications, including in endometrial, glioblastoma, lung and other cancers where Karyopharm is currently conducting clinical studies."

Commenting on additional milestones achieved in the third quarter of 2020, Michael G. Kauffman, MD, PhD, Chief Executive Officer of Karyopharm added, "Karyopharm achieved another strong quarter for XPOVIO sales, which grew approximately 15% compared to the second quarter of 2020. Sales growth was driven primarily by an increase in new multiple myeloma and diffuse large B-cell lymphoma (DLBCL) patient starts. Looking forward, we plan to continue our ongoing support for our supplemental New Drug Application (sNDA) seeking approval for XPOVIO as a treatment for patients with multiple myeloma after at least one prior line of therapy, for which the FDA has assigned a target Prescription Drug User Fee Act (PDUFA) action date of March 19, 2021."

## Third Quarter 2020 and Recent Highlights

### *XPOVIO in Multiple Myeloma and DLBCL*

- **XPOVIO U.S. Commercialization.** Oral XPOVIO became commercially available to patients with penta-refractory multiple myeloma in July 2019 and to patients with relapsed or refractory DLBCL in June 2020. During the third quarter of 2020, XPOVIO generated net product sales of \$21.3 million, representing a 15% increase compared to the second quarter of 2020. XPOVIO sales growth was primarily driven by an increase in new multiple myeloma patient starts compared to the second quarter of 2020. XPOVIO sales also benefited from the initial commercial launch in patients with relapsed or refractory DLBCL. In the third quarter of 2020, approximately 1,100 XPOVIO prescriptions were filled, which represented the highest quarterly level to date and was 15% higher than in the second quarter of 2020. Over 200 new physician prescribing accounts were added in the third quarter of 2020, which included both myeloma and DLBCL treating physicians. Finally, based on data from specialty pharmacies, prescription refill rates for XPOVIO continued to grow with the average number of prescriptions per patient reaching 2.9 by the end of September 2020, compared to 2.0 at the end of December 2019.
- **FDA Accepts sNDA Seeking Expanded Indication For Patients with Previously Treated Multiple Myeloma.** The FDA assigned a PDUFA action date of March 19, 2021 for Karyopharm's sNDA seeking approval for XPOVIO for the treatment of adult patients with multiple myeloma after at least one line of prior therapy. If approved, the Company expects to launch the expanded indication immediately thereafter. The sNDA is supported by the positive data from the pivotal Phase 3 BOSTON study, which was reported at the American Society of Clinical Oncology (ASCO) 2020 annual meeting. The BOSTON study evaluated once-weekly XPOVIO in combination with once-weekly Velcade® (bortezomib) and low-dose dexamethasone (SVd) compared to standard twice-weekly Velcade plus low-dose dexamethasone (Vd) in patients with multiple myeloma who have received one to three prior lines of therapy. The BOSTON study met its primary endpoint with a significant increase in median progression-free survival (PFS) in patients with multiple myeloma following 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=0.0075). There were no new safety signals on the SVd arm, and deaths were numerically lower on the SVd arm (N=47) as compared with the Vd arm (N=62).
- **Regulatory Strategy Update in Europe.** In January 2019, Karyopharm submitted a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) requesting conditional approval for XPOVIO in combination with dexamethasone as a 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 CHMP's outstanding questions, primarily related to 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 delayed the

review timelines in Europe. Karyopharm submitted the final requested re-monitoring data in September 2020 and in October 2020, Karyopharm received a further updated list of outstanding issues from the CHMP summarizing the remaining topics for Karyopharm to address and indicating that the CHMP intends to consult its Scientific Advisory Group for additional advice [in the fourth quarter of 2020]. Karyopharm continues to expect to receive an opinion from CHMP with respect to the MAA before the end of 2020. In addition, contingent upon and following receipt of CHMP's opinion, we expect to submit a MAA based on data from the BOSTON study before the end of 2020.

#### *XPOVIO in Development for Solid Tumors*

- **Twice-Weekly XPOVIO Demonstrates a Statistically Significant Reduction in the Risk of Disease Progression or Death Compared to Placebo; Hazard Ratio=0.70, p=0.023.** In November 2020, Karyopharm announced positive top-line results from the Phase 3 portion of the randomized, double blind, placebo controlled, cross-over, SEAL study evaluating single agent, oral XPOVIO versus placebo in patients with advanced unresectable dedifferentiated liposarcoma. The SEAL study met its primary endpoint of a statistically significant increase in PFS; hazard ratio=0.70; p=0.023. Among those patients who received XPOVIO, there was a trend towards an improvement in the median overall survival compared to those patients who began on the placebo arm of the study and never crossed over to the XPOVIO treatment arm. The safety profile for XPOVIO was consistent with previous clinical studies with fewer hematologic and infectious adverse events as compared to XPOVIO studies in patients with multiple myeloma and DLBCL. The clinical data from this study have been selected for an oral presentation at the CTOS Annual Meeting on November 20, 2020 at 10:30 AM ET. Additionally, Karyopharm intends to use the data from the SEAL study to submit an NDA to the FDA in the first quarter of 2021 requesting approval of XPOVIO to treat the patient population studied in SEAL.
- **Clinical Data from Advanced Solid Tumor Studies Presented at the European Society for Medical Oncology (ESMO) Virtual Congress.** In September 2020, encouraging clinical data from investigator-sponsored studies evaluating XPOVIO in combination with established cancer therapies were presented at ESMO. Clinical data from XPOVIO studies included: (i) combination data with pembrolizumab for the treatment of melanoma, (ii) combination data with carboplatin and paclitaxel for the treatment of advanced or metastatic solid tumors, and (iii) combination data with topotecan for the treatment of advanced or metastatic solid tumors. The Company believes that the encouraging results from these combination studies warrant further research into the potential utility of XPOVIO in solid tumors and will help Karyopharm further prioritize future clinical development activities.

#### *Low Dose Selinexor in Development for COVID-19*

- **Presented Phase 2 Data at COVID-19 Focused Medical Meeting.** Selinexor data was highlighted recently in an oral presentation at the International Society for Influenza and Other Respiratory Virus Diseases Antiviral Group (ISIRV-AVG) Virtual Conference on Therapeutics for COVID-19. While the results of the Phase 2 study demonstrated encouraging anti-viral and anti-inflammatory activity in an important subset of treated patients, the trial was discontinued following an interim analysis which indicated that the trial was unlikely to meet its pre-specified primary endpoint. While the FDA's opinion was that the benefit-risk ratio for this study was not favorable, Karyopharm is encouraged by the potential mechanistic activity of XPO1 inhibition and believes these results warrant further research of XPO1 inhibitors in COVID-19 and other infectious diseases.

#### *Corporate Updates*

- **Sharon Shacham, Winner of EY Entrepreneur of the Year® 2020 New England Award.** In October 2020, Karyopharm's founder, Dr. Sharon Shacham, was selected as a winner in the EY Entrepreneur of the Year 2020 New England Awards Program. For more than 30 years, this award has served as one of the world's most prestigious business awards recognizing entrepreneurs who have disrupted industries, created new product categories and successfully brought innovations that have transformed our world. Dr. Shacham was recognized for her scientific research that led to the development and FDA approval of XPOVIO, as well as for leading Karyopharm from its inception to what is now a global pharmaceutical company focused on the discovery, development, and commercialization of novel medicines for patients with cancer and other major diseases.
- **Christy J. Oliger Appointed to the Board.** In August 2020, Karyopharm appointed Christy J. Oliger to its Board of Directors. Ms. Oliger is the former Senior Vice President of the Oncology Business Unit at Genentech, a leading biotechnology company dedicated to pursuing groundbreaking science to discover and develop medicines for people with serious and life-threatening diseases. Ms. Oliger served in a wide variety of commercial leadership positions at Genentech from 2000 until 2020. Ms. Oliger's strategic expertise and counsel will be critical to Karyopharm as it continues to expand the global development and commercialization of XPOVIO into new patient populations and potential new indications.

#### **Third Quarter 2020 Financial Results**

**Net product revenue:** Net product revenue for the third quarter of 2020 was \$21.3 million, compared to \$12.8 million for the third quarter of 2019.

**License and other revenue:** License and other revenue for the third quarter of 2020 was negligible, compared to \$0.3 million for the third quarter of 2019.

**Cost of sales:** Cost of sales totaled \$0.4 million for the third quarter of 2020, compared to \$1.0 million for the third quarter of 2019. Cost of sales reflects the costs of XPOVIO units sold and third-party royalties on net product revenue.

**Research and development (R&D) expenses:** R&D expenses for the third quarter of 2020 were \$37.0 million, compared to \$26.3 million for the third quarter of 2019. The increase in R&D expenses compared to the third quarter of last year was primarily attributable to COVID-19 trial activity and continued activity in our other ongoing clinical trials.

**Selling, general and administrative (SG&A) expenses:** For the third quarter of 2020, SG&A expenses were \$31.0 million, compared to \$25.3 million for the third quarter of 2019. The increase in SG&A expenses compared to the third quarter of last year was due primarily to activities to support the U.S. commercialization of XPOVIO, including the launch of XPOVIO as a treatment for patients with relapsed or

refractory DLBCL.

**Interest expense:** Interest expense for the third quarter of 2020 was \$6.8 million, compared to \$3.1 million for the third quarter of 2019. The increase in interest expense was primarily attributable to the imputed interest on the deferred royalty obligation Karyopharm has with HealthCare Royalty Partners.

**Net loss:** Karyopharm reported a net loss of \$53.5 million, or \$0.73 per share, for the third quarter of 2020, compared to a net loss of \$41.4 million, or \$0.67 per share, for the third quarter of 2019. Net loss included non-cash stock-based compensation expense of \$6.5 million and \$3.7 million for the third quarters of 2020 and 2019, respectively.

**Cash position:** Cash, cash equivalents, restricted cash and investments as of September 30, 2020 totaled \$304.2 million, compared to \$265.8 million as of December 31, 2019.

## 2020 Financial Outlook

Based on its current operating plans, Karyopharm continues to expect its non-GAAP R&D and SG&A expenses, which excludes stock-based compensation expense, for the full year 2020 to be in the range of \$240.0 million to \$260.0 million. 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 second half 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, Monday, November 2, 2020, at 4:30 p.m. Eastern Time, to discuss the third quarter 2020 financial results, recent accomplishments, clinical developments and business plans. To access the conference call, please dial (877) 870-4263 (local) or (412) 317-0790 (international) at least 10 minutes prior to the start time and ask to be joined into the Karyopharm Therapeutics call. 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 XPOVIO® (selinexor)

XPOVIO is a first-in-class, oral Selective Inhibitor of Nuclear Export (SINE) compound. XPOVIO functions by selectively binding to and inhibiting the nuclear export protein exportin 1 (XPO1, also called CRM1). XPOVIO blocks the nuclear export of tumor suppressor, growth regulatory and anti-inflammatory proteins, leading to accumulation of these proteins in the nucleus and enhancing their anti-cancer activity in the cell. The forced nuclear retention of these proteins can counteract a multitude of the oncogenic pathways that, unchecked, allow cancer cells with severe DNA damage to continue to grow and divide in an unrestrained fashion. Karyopharm received accelerated U.S. Food and Drug Administration (FDA) approval of XPOVIO in July 2019 in combination with dexamethasone for the treatment of adult patients with relapsed refractory multiple myeloma (RRMM) 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. Karyopharm has also submitted a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) with a request for conditional approval of selinexor in this same RRMM indication. Karyopharm's supplemental New Drug Application (sNDA) requesting an expansion of its current indication to include the treatment for patients with multiple myeloma after at least one prior line of therapy has been accepted for filing by the FDA. In June 2020, Karyopharm received accelerated FDA approval of XPOVIO for its second indication in adult patients with relapsed or refractory diffuse large B-cell lymphoma (DLBCL), not otherwise specified, including DLBCL arising from follicular lymphoma, after at least 2 lines of systemic therapy. Selinexor is also being evaluated in several other mid-and later-phase clinical trials across multiple cancer indications, including as a potential backbone therapy in combination with approved myeloma therapies (STOMP), in liposarcoma (SEAL) and 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. Additional clinical trial information for selinexor is available at [www.clinicaltrials.gov](http://www.clinicaltrials.gov).

For more information about Karyopharm's products or clinical trials, please contact the Medical Information department at:

Tel: +1 (888) 209-9326

Email: [medicalinformation@karyopharm.com](mailto:medicalinformation@karyopharm.com)

## IMPORTANT SAFETY INFORMATION

**Thrombocytopenia:** XPOVIO can cause life-threatening thrombocytopenia, potentially leading to hemorrhage. Thrombocytopenia was reported in patients with multiple myeloma (MM) and developed or worsened in patients with DLBCL.

Thrombocytopenia is the leading cause of dosage modifications. Monitor platelet counts at baseline and throughout treatment. Monitor more frequently during the first 3 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, reduce dose, or permanently discontinue based on severity of adverse reaction.

**Neutropenia:** XPOVIO can cause life-threatening neutropenia, potentially increasing the risk of infection. Neutropenia and febrile neutropenia occurred in patients with MM and in patients with DLBCL.

Obtain white blood cell counts with differential at baseline and throughout treatment. Monitor more frequently during the first 3 months of treatment. Monitor patients for signs and symptoms of concomitant infection and evaluate promptly. Consider supportive measures, including antimicrobials and growth factors (e.g., G-CSF). Interrupt, reduce dose, or permanently discontinue based on severity of adverse reaction (AR).

**Gastrointestinal Toxicity:** XPOVIO can cause severe gastrointestinal toxicities in patients with MM and DLBCL.

**Nausea/Vomiting:** Provide prophylactic antiemetics. Administer 5-HT<sub>3</sub> receptor antagonists and other anti-nausea agents prior to and during treatment with XPOVIO. Interrupt, reduce dose, or permanently discontinue based on severity of ARs. Administer intravenous fluids to prevent dehydration and replace electrolytes as clinically indicated.

**Diarrhea:** Interrupt, reduce dose, or permanently discontinue based on severity of ARs. Provide standard anti-diarrheal agents, administer intravenous fluids to prevent dehydration, and replace electrolytes as clinically indicated.

**Anorexia/Weight Loss:** Monitor weight, nutritional status, and volume status at baseline and throughout treatment. Monitor more frequently during the first 3 months of treatment. Interrupt, reduce dose, or permanently discontinue based on severity of ARs. Provide nutritional support, fluids, and electrolyte repletion as clinically indicated.

**Hyponatremia:** XPOVIO can cause severe or life-threatening hyponatremia. Hyponatremia developed in patients with MM and in patients with DLBCL.

Monitor sodium level at baseline and throughout treatment. Monitor more frequently during the first 2 months of treatment. Correct sodium levels for concurrent hyperglycemia (serum glucose >150 mg/dL) and high serum paraprotein levels. Assess hydration status and manage hyponatremia per clinical guidelines, including intravenous saline and/or salt tablets as appropriate and dietary review. Interrupt, reduce dose, or permanently discontinue based on severity of the AR.

**Serious Infection:** XPOVIO can cause serious and fatal infections. Most infections were not associated with Grade 3 or higher neutropenia. Atypical infections reported after taking XPOVIO include, but are not limited to, fungal pneumonia and herpesvirus infection.

Monitor for signs and symptoms of infection, and evaluate and treat promptly.

**Neurological Toxicity:** XPOVIO can cause life-threatening neurological toxicities.

Coadministration of XPOVIO with other products that cause dizziness or mental status changes may increase the risk of neurological toxicity.

Advise patients to refrain from driving and engaging in hazardous occupations or activities, such as operating heavy or potentially dangerous machinery, until the neurological toxicity fully resolves. Optimize hydration status, hemoglobin level, and concomitant medications to avoid exacerbating dizziness or mental status changes. Institute fall precautions as appropriate.

**Embryo-Fetal Toxicity:** XPOVIO can cause fetal harm when administered to a pregnant woman.

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 (ARs) in ≥20% of patients with MM are thrombocytopenia, fatigue, nausea, anemia, decreased appetite, decreased weight, diarrhea, vomiting, hyponatremia, neutropenia, leukopenia, constipation, dyspnea, and upper respiratory tract infection.

The most common ARs, excluding laboratory abnormalities, in ≥20% of patients with DLBCL are fatigue, nausea, diarrhea, appetite decrease, weight decrease, constipation, vomiting, and pyrexia. Grade 3-4 laboratory abnormalities in ≥15% of patients included thrombocytopenia, lymphopenia, neutropenia, anemia, and hyponatremia. Grade 4 laboratory abnormalities in ≥5% were thrombocytopenia, lymphopenia, and neutropenia.

In patients with MM, fatal ARs occurred in 9% of patients. Serious ARs occurred in 58% of patients. Treatment discontinuation rate due to ARs was 27%. The most frequent ARs requiring permanent discontinuation in ≥4% of patients included fatigue, nausea, and thrombocytopenia.

In patients with DLBCL, fatal ARs occurred in 3.7% of patients within 30 days, and 5% of patients within 60 days of last treatment; the most frequent fatal AR was infection (4.5% of patients). Serious ARs occurred in 46% of patients; the most frequent serious AR was infection. Discontinuation due to ARs occurred in 17% of patients.

## USE IN SPECIFIC POPULATIONS

In MM, no overall difference in effectiveness of XPOVIO was observed in patients >65 years old when compared with younger patients. Patients ≥75 years old had a higher incidence of discontinuation due to an AR than younger patients, a higher incidence of serious ARs, and a higher incidence of fatal ARs.

Clinical studies in patients with relapsed or refractory DLBCL did not include sufficient numbers of patients aged 65 and over to determine whether they respond differently from younger patients.

The effect of end-stage renal disease ( $CL_{CR} < 15$  mL/min) or hemodialysis on XPOVIO pharmacokinetics is unknown.

**To report SUSPECTED ADVERSE REACTIONS, contact Karyopharm Therapeutics Inc. at 1-888-209-9326 or FDA at 1-800-FDA-1088 or [www.fda.gov/medwatch](http://www.fda.gov/medwatch).**

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

### **About Karyopharm Therapeutics**

Karyopharm Therapeutics Inc. (Nasdaq: KPTI) is a commercial-stage pharmaceutical company pioneering novel cancer therapies and 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® (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. In June 2020, XPOVIO was approved by the FDA as a treatment for patients with relapsed or refractory diffuse large B-cell lymphoma. A Marketing Authorization Application for selinexor for patients with heavily pretreated multiple myeloma 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](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 XPOVIO for the treatment of patients with relapsed or refractory 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 Company's regulatory strategy, 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 expected design of the Company's clinical trials; the therapeutic potential of and potential clinical development plans for Karyopharm's drug candidates, especially selinexor; Karyopharm's collaboration efforts with third-parties; 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 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 negatively impacting 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 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 Quarterly Report on Form 10-Q for the quarter ended June 30, 2020, which was filed with the Securities and Exchange Commission (SEC) on August 4, 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.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Revenues:				
Product revenue, net	\$ 21,330	\$ 12,821	\$ 55,992	\$ 12,821
License and other revenue	3	328	16,993	9,976
Total revenues	<u>21,333</u>	<u>13,149</u>	<u>72,985</u>	<u>22,797</u>
Operating expenses:				
Cost of sales	438	1,013	1,653	1,013
Research and development	37,037	26,270	113,628	90,761
Selling, general and administrative	30,967	25,267	92,488	77,032
Total operating expenses	<u>68,442</u>	<u>52,550</u>	<u>207,769</u>	<u>168,806</u>
Loss from operations	(47,109)	(39,401)	(134,784)	(146,009)
Other income (expense):				
Interest income	600	1,137	2,424	4,320
Interest expense	(6,801)	(3,093)	(20,068)	(9,180)
Other (expense) income, net	(141)	10	(177)	(36)
Total other expense, net	<u>(6,342)</u>	<u>(1,946)</u>	<u>(17,821)</u>	<u>(4,896)</u>
Loss before income taxes	(53,451)	(41,347)	(152,605)	(150,905)
Income tax provision	(44)	(20)	(247)	(38)
Net loss	<u>\$ (53,495)</u>	<u>\$ (41,367)</u>	<u>\$ (152,852)</u>	<u>\$ (150,943)</u>
Net loss per share—basic and diluted	<u>\$ (0.73)</u>	<u>\$ (0.67)</u>	<u>\$ (2.14)</u>	<u>\$ (2.46)</u>
Weighted-average number of common shares outstanding used in net loss per share—basic and diluted	<u>73,466</u>	<u>62,093</u>	<u>71,479</u>	<u>61,297</u>

**KARYOPHARM THERAPEUTICS INC.**  
**CONDENSED CONSOLIDATED BALANCE SHEETS**  
(unaudited)  
(in thousands)

	September 30, 2020	December 31, 2019
<b>Assets</b>		
Cash, cash equivalents and investments	\$ 302,415	\$ 263,972
Restricted cash	1,806	1,831
Accounts receivable	11,062	7,862
Property and equipment, net	2,357	3,046
Other assets	19,947	18,252
Total assets	<u>\$ 337,587</u>	<u>\$ 294,963</u>
<b>Liabilities and stockholders' equity</b>		
Deferred revenue	\$ 297	\$ 4,533
Convertible senior notes	115,802	109,857
Deferred royalty obligation	73,588	73,588
Other liabilities	64,130	57,211
Total liabilities	<u>253,817</u>	<u>245,189</u>
Total stockholders' equity	<u>83,770</u>	<u>49,774</u>
Total liabilities and stockholders' equity; 73,528 and 65,370 shares issued and outstanding at September 30, 2020 and December 31, 2019, respectively	<u>\$ 337,587</u>	<u>\$ 294,963</u>

SOURCE Karyopharm Therapeutics Inc.

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<https://investors.karyopharm.com/2020-11-02-Karyopharm-Reports-Third-Quarter-2020-Financial-Results-and-Highlights-Recent-Company-Progress>