

# Karyopharm Reports Fourth Quarter and Full Year 2020 Financial Results and Highlights Recent Company Progress

- Commercial Launch of XPOVIO® (selinexor) In Expanded Multiple Myeloma Indication Fully Underway Following December 18, 2020 FDA Approval --
- XPOVIO Net Product Sales of \$20.2 Million for Fourth Quarter 2020 and \$76.2 Million for the Year 2020; Total Revenues of \$35.1 Million for the Fourth Quarter 2020 and \$108.1 Million for the Year 2020 --
- Positive CHMP Opinion for NEXPOVIO® (selinexor) in Penta-Refractory Multiple Myeloma Issued; European Commission Decision Expected by April 2021 --
- Conference Call Scheduled for Today at 8:30 a.m. ET --

NEWTON, Mass., Feb. 11, 2021 /PRNewswire/ -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a commercial-stage pharmaceutical company pioneering novel cancer therapies, today reported financial results for the fourth quarter and full year ended December 31, 2020. In addition, Karyopharm highlighted select corporate milestones, including details regarding the ongoing U.S. commercialization of XPOVIO® (selinexor), regulatory progress in Europe, and provided an overview of its key clinical development programs.

"Karyopharm made substantial progress in 2020 towards its mission of improving the lives of patients with cancer, marked by the FDA approval of XPOVIO in two additional oncology indications: relapsed or refractory diffuse large B-cell lymphoma (DLBCL) and multiple myeloma in patients who have received at least one prior therapy," said Michael G. Kauffman, MD, PhD, Chief Executive Officer of Karyopharm. "In addition, we were particularly encouraged that the National Comprehensive Cancer Network® (NCCN) added three different XPOVIO combination regimens to its Clinical Practice Guidelines in Oncology (NCCN Guidelines®) for previously treated multiple myeloma. As we begin to execute our plans for 2021, we are focused on the commercial expansion of XPOVIO into the second- and third-line treatment settings for multiple myeloma, a significantly increased addressable patient population. We are also progressing the international expansion of NEXPOVIO® (selinexor), with a European Commission (EC) regulatory decision expected by April 2021 following the recent positive opinion issued from the Committee for Medicinal Products for Human Use (CHMP). In parallel to our commercial efforts in the hematology space, our clinical development of XPOVIO in solid tumor indications continues to advance, with top-line data from the Phase 3 SIENDO study in endometrial cancer expected in the second half of 2021."

## Fourth Quarter 2020 and Recent Highlights

### *XPOVIO in Multiple Myeloma and DLBCL*

- **XPOVIO Receives Early Approval from the FDA for Patients with Multiple Myeloma After At Least One Prior Therapy.** On December 18, 2020, the FDA approved once-weekly, oral XPOVIO in combination with once-weekly Velcade® (bortezomib) and dexamethasone for the treatment of adult patients with multiple myeloma who have received at least one prior therapy, which became commercially available in the U.S. immediately following approval. Karyopharm is leveraging its existing commercial infrastructure to market this third oncology indication, which significantly expands the addressable patient population to the 2<sup>nd</sup> and 3<sup>rd</sup> line treatment settings and extends the anticipated duration of treatment as compared to patients currently receiving XPOVIO in the penta-refractory treatment setting.
- **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 fourth quarter of 2020, XPOVIO generated net product sales of \$20.2 million, representing a 5% decrease compared to the third quarter of 2020. Net sales for the fourth quarter were largely driven by prescription demand from both academic and community-based oncologists for patients with penta-refractory multiple myeloma. Sales were affected by the surge in U.S. COVID-19 cases late in 2020 impacting both patient visits to their healthcare providers, as well as reduced in-person access for Karyopharm's commercial team to its physician customers. Additionally, increased competition, specifically in the penta-refractory multiple myeloma setting, also contributed to the sales pressure in the quarter.

In the fourth quarter of 2020, approximately 1,000 XPOVIO prescriptions were filled, with prescription demand higher in December 2020 compared to either October or November 2020. Over 170 new physician prescribing accounts were added in the fourth quarter of 2020, which included both myeloma and DLBCL treating physicians. Finally, based on data from specialty pharmacies, prescription refill rates for XPOVIO remained consistent compared to the third quarter of 2020 with the average number of prescriptions per patient estimated at 2.9 by the end of December 2020, compared to 2.0 at the end of December 2019.

On a quarterly basis, Karyopharm expects XPOVIO sales to return to growth beginning in the first quarter of 2021, compared to the fourth quarter of 2020, following the expanded U.S. FDA approval of XPOVIO as a treatment for patients

with multiple myeloma after at least one prior therapy.

- **Three XPOVIO Treatment Regimens Added to National Comprehensive Cancer Network® Guidelines.** In December 2020, the NCCN added three different XPOVIO combination regimens to its Clinical Practice Guidelines in Oncology for previously treated multiple myeloma. The XPOVIO regimens added to the NCCN guidelines include: (i) selinexor / bortezomib / dexamethasone (once-weekly); (ii) selinexor / daratumumab / dexamethasone; and (iii) selinexor / pomalidomide / dexamethasone, which is an all-oral treatment regimen. The NCCN Guidelines are a comprehensive set of guidelines detailing the sequential management decisions and interventions that currently apply to 97% of cancers affecting patients in the U.S. and are intended to ensure that all patients receive preventive, diagnostic, treatment, and supportive services that will most likely lead to optimal outcomes.
- **Positive CHMP Opinion for NEXPOVIO® (selinexor) for Multiple Myeloma.** In January 2021, the European Medicines Agency's (EMA) CHMP adopted a positive opinion recommending the conditional approval for NEXPOVIO (the expected brand name for selinexor in Europe) in combination with dexamethasone for the treatment of multiple myeloma in adult patients who have received at least four prior therapies and whose disease is refractory to at least two proteasome inhibitors, two immunomodulatory agents, and an anti-CD38 monoclonal antibody, and who have demonstrated disease progression on the last therapy. The Company expects a final decision from the EC on its NEXPOVIO marketing authorization application by April 2021. Karyopharm also intends to submit a second regulatory filing to the EMA (Type II variation) based on the data from the confirmatory Phase 3 BOSTON study by April 2021.
- **First Patient Dosed in Phase 2/3 Confirmatory Study in DLBCL** In February 2021, the first patient was dosed in the Phase 2/3 XPORT-DLBCL-030 study of XPOVIO in patients with DLBCL. As part of XPOVIO's accelerated approval in DLBCL, XPORT-DLBCL-030 study will serve as the confirmatory study for this indication. This study will assess the effect of XPOVIO or placebo added to a standard backbone immunochemotherapy of rituximab gemcitabine-dexamethasone-platinum (R-GDP) in patients who have had one to three prior treatments for DLBCL.
- **Myeloma and DLBCL Data Presented at the American Society of Hematology (ASH) 2020 Annual Meeting** Multiple data presentations highlighting XPOVIO were presented at the ASH 2020 Annual Meeting held December 5-8, 2020 and continue to reinforce the broad potential clinical utility of XPOVIO. This included updated data from the Pomalyst® (pomalidomide), Kyprolis® (carfilzomib) and Revlimid® (lenalidomide) arms of the Phase 1b/2 STOMP study evaluating XPOVIO in combination with backbone therapies in patients with relapsed or refractory multiple myeloma, which continued to demonstrate favorable response rates and durability. Several new subgroup analyses from the Phase 3 BOSTON study were also presented and demonstrated XPOVIO is effective and well tolerated regardless of prior lines of treatment, including prior treatment with a proteasome inhibitor or lenalidomide, and across several important patient subgroups, including those with high risk cytogenetics, the elderly and the frail. A subgroup analyses of the Phase 2b SADAL study in DLBCL were also presented that highlighted XPOVIO's positive efficacy and safety in patients stratified by age and renal function at baseline.
- **Phase 3 BOSTON Study Results Published in *The Lancet*.** In November 2020, the results of the Phase 3 BOSTON study evaluating XPOVIO in patients with relapsed or refractory multiple myeloma were published in [The Lancet](#). The BOSTON study evaluated once-weekly XPOVIO in combination with once-weekly Velcade® and low-dose dexamethasone (XVd) against standard twice-weekly Velcade® plus dexamethasone (Vd) in adult patients with multiple myeloma who had received one to three prior lines of therapy. The BOSTON study met its primary endpoint with a median progression-free survival (PFS) in the XVd arm of 13.93 months compared to 9.46 months in the Vd arm, representing a 4.47 month (47%) increase in median PFS (hazard ratio HR=0.70; p=0.0075). The XVd group also demonstrated a significantly greater overall response rate compared to the Vd group (76.4% vs. 62.3%, p=0.0012). Additionally, peripheral neuropathy rates were significantly lower on the XVd arm compared to the Vd arm (32.3% vs. 47.1%; p=0.0010).

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

- **Phase 3 SIENDO Study in Endometrial Cancer Passes Interim Futility Analysis** In November 2020, Karyopharm announced that, following a pre-specified interim futility analysis for the ongoing Phase 3 SIENDO study, the Data and Safety Monitoring Board (DSMB) recommended that the study should continue as planned without the need to add additional patients to the trial or to amend the study protocol. The SIENDO study is an ongoing multicenter, blinded, placebo-controlled, randomized Phase 3 study evaluating the efficacy and safety for front-line maintenance therapy with XPOVIO in patients with advanced or recurrent endometrial cancer. Participants with primary stage IV or recurrent disease who had a partial or complete response after a single line of at least 12 weeks of standard taxane-platinum combination chemotherapy are randomized in a 2:1 manner to receive either maintenance therapy of 80mg of XPOVIO taken once per week or placebo, until disease progression. Top-line data from the SIENDO study is expected in the second half of 2021.
- **Positive Phase 3 SEAL Data Evaluating XPOVIO in Liposarcoma Presented.** In November 2020, positive 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 were presented at the Connective Tissue Oncology Society (CTOS) 2020 Annual Meeting. The SEAL study met its primary endpoint of a

statistically significant increase in median PFS. The median PFS in the XPOVIO arm of the Phase 3 portion of the SEAL study was 2.83 months compared to 2.07 months in the placebo arm (HR=0.70; p=0.023). These data indicate that treatment with XPOVIO reduced the risk of disease progression or death by approximately 30%, compared to placebo. The estimated 6-month PFS survival probability was 23.9% on the XPOVIO arm compared to 13.9% on placebo. Additionally, the 12-month PFS survival probability was 8.4% on the XPOVIO arm compared to 2% on the placebo arm. Karyopharm no longer intends to submit a New Drug Application to the FDA in the first quarter of 2021 and plans to submit the results for publication and apply for compendia listings for this very uncommon cancer.

### *Corporate Updates*

- **Continued Progress Towards the International Expansion of XPOVIO.** In February 2021, the Israeli Ministry of Health, Israel's regulatory agency responsible for the approval of new medicines, issued a principal approval letter for XPOVIO as a treatment for patients with either multiple myeloma or diffuse large B-cell lymphoma. Additionally, in December 2020, Karyopharm signed an exclusive distribution agreement for the commercialization of XPOVIO in Canada with FORUS Therapeutics Inc. Under the terms of the agreement, Karyopharm received an upfront payment of \$5.0 million and is eligible to receive additional payments if certain prespecified regulatory and commercial milestones are achieved, as well as double-digit royalties on future net sales of XPOVIO in Canada. Separately, Karyopharm's partner in the Asia-Pacific region, Antengene Therapeutics Limited, filed for regulatory approval of selinexor in both multiple myeloma and DLBCL indications in Australia, Singapore and South Korea in December 2020, which triggered approximately \$10.0 million in milestone payments to Karyopharm.
- **New Appointment to Board of Directors.** In December 2020, Karyopharm appointed Chen Schor, MBA, to its Board of Directors. Mr. Schor has led biotechnology companies across all stages, from formation and early stage discovery to leading a publicly traded multi-product company with significant external partnerships. He currently serves as President and Chief Executive Officer and a member of the board of directors of Adicet Bio, Inc.

### **Full Year and Fourth Quarter 2020 Financial Results**

**Net product revenue:** Net product revenue for the fourth quarter of 2020 was \$20.2 million, compared to \$17.7 million for the fourth quarter of 2019. Net product revenue for the year ended December 31, 2020 was \$76.2 million, compared to \$30.5 million for the year ended 2019.

**License and other revenue:** License and other revenue for the fourth quarter of 2020 was \$14.9 million, compared to \$0.4 million for the fourth quarter of 2019. This increase was driven by approximately \$10.0 million in milestone payments associated with regulatory filings in Asia from Antengene Therapeutics Limited, as well as a \$5.0 million upfront payment from FORUS Therapeutics Inc. upon the execution of a commercial distribution agreement for Canada. License and other revenue in 2020 were \$31.9 million, compared to \$10.4 million in 2019.

**Cost of sales:** Cost of sales for the fourth quarter of 2020 were \$1.1 million, compared to \$1.4 million for the fourth quarter of 2019. Cost of sales for the year ended December 31, 2020 were \$2.7 million, compared to \$2.4 million for the year ended December 31, 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 fourth quarter of 2020 were \$37.2 million, compared to \$31.6 million for the fourth quarter of 2019. R&D expenses for 2020 were \$150.8 million, compared to \$122.3 million for 2019. The increase in R&D expenses in 2020 compared to 2019 was primarily attributable to costs incurred related to our COVID-19 trial activity, which are not expected to be incurred in 2021, and continued activity in our other ongoing clinical trials.

**Selling, general and administrative (SG&A) expenses:** SG&A expenses for the fourth quarter of 2020 were \$33.9 million, compared to \$28.4 million for the fourth quarter of 2019. SG&A expenses for the year ended December 31, 2020 were \$126.4 million, compared to \$105.4 million for the year ended December 31, 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, including the launches of XPOVIO as a treatment for patients with relapsed or refractory DLBCL and in combination with once-weekly Velcade® and dexamethasone for the treatment of adult patients with multiple myeloma who have received at least one prior therapy.

**Interest expense:** Interest expense for the fourth quarter of 2020 was \$7.1 million, compared to \$6.5 million for the fourth quarter of 2019. Interest expense for the full year 2020 was \$27.1 million, compared to \$15.6 million for the full year 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 \$43.4 million, or \$0.59 per share, for the fourth quarter of 2020, compared to a net loss of \$48.6 million, or \$0.76 per share, for the fourth quarter of 2019. Net loss includes non-cash stock-based compensation expense of \$6.3 million and \$3.6 million for the fourth quarter of 2020 and 2019, respectively. Karyopharm reported a net loss of \$196.3 million, or \$2.72 per share, for the year ended December 31, 2020, compared to a net loss of \$199.6 million, or \$3.22 per share, for the year ended December 31, 2019. Net loss includes non-cash stock-based compensation expense of \$24.4 million and \$15.3 million for the years ended December 31, 2020 and 2019, respectively.

**Cash position:** Cash, cash equivalents, restricted cash and investments as of December 31, 2020 totaled \$276.7 million, compared to \$265.8 million as of December 31, 2019.

## 2021 Financial Outlook

Based on its current operating plans, Karyopharm expects its non-GAAP R&D and SG&A expenses, excluding stock-based compensation expense, for the year ending December 31, 2021 to be in the range of \$280 million to \$300 million.

The Company expects that its existing cash, cash equivalents and investments, and the revenue it expects to generate from XPOVIO product sales, as well as revenue generated from its license agreements, will be sufficient to fund its planned operations into late 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, Thursday, February 11, 2021, at 8:30 a.m. Eastern Time, to discuss the fourth quarter and full year 2020 financial results, recent accomplishments, clinical developments and business plans. To access the conference call, please dial (888) 349-0102 (local) or (412) 902-4299 (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 indication to include the treatment for patients with multiple myeloma after at least one prior therapy was approved by the FDA on December 18, 2020. 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)

XPOVIO® (selinexor) is a prescription medicine approved:

- In combination with bortezomib and dexamethasone for the treatment of adult patients with multiple myeloma who have received at least one prior therapy (XVd).
- In combination with dexamethasone for the treatment of adult patients with relapsed or refractory multiple myeloma who have received at least four prior therapies and whose disease is refractory to at least two proteasome inhibitors, at least

two immunomodulatory agents, and an anti-CD38 monoclonal antibody (Xd).

- For the treatment of 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. This indication is approved under accelerated approval based on response rate. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trial(s).

## SELECT IMPORTANT SAFETY INFORMATION

### Warnings and Precautions

- **Thrombocytopenia:** Monitor platelet counts throughout treatment. Manage with dose interruption and/or reduction and supportive care.
- **Neutropenia:** Monitor neutrophil counts throughout treatment. Manage with dose interruption and/or reduction and granulocyte colony-stimulating factors.
- **Gastrointestinal Toxicity:** Nausea, vomiting, diarrhea, anorexia, and weight loss may occur. Provide antiemetic prophylaxis. Manage with dose interruption and/or reduction, antiemetics, and supportive care.
- **Hyponatremia:** Monitor serum sodium levels throughout treatment. Correct for concurrent hyperglycemia and high serum paraprotein levels. Manage with dose interruption, reduction, or discontinuation, and supportive care.
- **Serious Infection:** Monitor for infection and treat promptly.
- **Neurological Toxicity:** Advise patients to refrain from driving and engaging in hazardous occupations or activities until neurological toxicity resolves. Optimize hydration status and concomitant medications to avoid dizziness or mental status changes.
- **Embryo-Fetal Toxicity:** Can cause fetal harm. Advise females of reproductive potential and males with a female partner of reproductive potential, of the potential risk to a fetus and use of effective contraception.
- **Cataract:** Cataracts may develop or progress. Treatment of cataracts usually requires surgical removal of the cataract.

### Adverse Reactions

- The most common adverse reactions ( $\geq 20\%$ ) in patients with multiple myeloma who receive XVd are fatigue, nausea, decreased appetite, diarrhea, peripheral neuropathy, upper respiratory tract infection, decreased weight, cataract and vomiting. Grade 3–4 laboratory abnormalities ( $\geq 10\%$ ) are thrombocytopenia, lymphopenia, hypophosphatemia, anemia, hyponatremia and neutropenia. In the BOSTON trial, fatal adverse reactions occurred in 6% of patients within 30 days of last treatment. Serious adverse reactions occurred in 52% of patients. Treatment discontinuation rate due to adverse reactions was 19%.
- The most common adverse reactions ( $\geq 20\%$ ) in patients with multiple myeloma who receive Xd are thrombocytopenia, fatigue, nausea, anemia, decreased appetite, decreased weight, diarrhea, vomiting, hyponatremia, neutropenia, leukopenia, constipation, dyspnea and upper respiratory tract infection. In the STORM trial, fatal adverse reactions occurred in 9% of patients. Serious adverse reactions occurred in 58% of patients. Treatment discontinuation rate due to adverse reactions was 27%.
- The most common adverse reactions (incidence  $\geq 20\%$ ) in patients with DLBCL, excluding laboratory abnormalities, are fatigue, nausea, diarrhea, appetite decrease, weight decrease, constipation, vomiting, and pyrexia. Grade 3–4 laboratory abnormalities ( $\geq 15\%$ ) are thrombocytopenia, lymphopenia, neutropenia, anemia, and hyponatremia. In the SADAL trial, fatal adverse reactions occurred in 3.7% of patients within 30 days, and 5% of patients within 60 days of last treatment; the most frequent fatal adverse reaction was infection (4.5% of patients). Serious adverse reactions occurred in 46% of patients; the most frequent serious adverse reaction was infection (21% of patients). Discontinuation due to adverse reactions occurred in 17% of patients.

### Use In Specific Populations

Lactation: Advise not to breastfeed.

For additional product information, including full prescribing information, please visit [www.XPOVIO.com](http://www.XPOVIO.com).

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).

### 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), is approved in the U.S. in multiple hematologic malignancy indications,

including in combination with Velcade® (bortezomib) and dexamethasone for the treatment of patients with multiple myeloma after at least one prior therapy, in combination with dexamethasone for the treatment of patients with heavily pretreated multiple myeloma and as a monotherapy for the treatment of patients with relapsed or refractory diffuse large B-cell lymphoma. A Marketing Authorization Application for NEXPOVIO® (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 guidance on its 2021 non-GAAP research and development and selling, general and administrative expenses; 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; and the therapeutic potential of and potential clinical development plans for Karyopharm's drug candidates, especially selinexor. Such statements are subject to numerous important factors, risks and uncertainties, many of which are beyond Karyopharm's control, that may cause actual events or results to differ materially from Karyopharm's current expectations. For example, there can be no guarantee that Karyopharm will successfully commercialize XPOVIO; that regulators will agree that selinexor qualifies for conditional approval in the European Union as a result of data from the STORM study or confirmatory approval in the European Union based on the BOSTON study in patients with 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 or regulatory approval of drug candidates by Karyopharm's competitors for products or product candidates in which Karyopharm is currently commercializing or developing; and Karyopharm's ability to obtain, maintain and enforce patent and other intellectual property protection for any of its products or product candidates. These and other risks are described under the caption "Risk Factors" in Karyopharm's Quarterly Report on Form 10-Q for the quarter ended September 30, 2020, which was filed with the Securities and Exchange Commission (SEC) on November 2, 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.

XPOVIO®(selinexor) is a registered trademark of Karyopharm Therapeutics Inc. Any other trademarks referred to in this presentation are the property of their respective owners.

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

Three Months Ended December 31,		Year Ended December 31,	
2020	2019	2020	2019

Revenues:				
Product revenue, net	\$ 20,218	\$ 17,719	\$ 76,210	\$ 30,540
License and other revenue	14,882	377	31,875	10,353
Total revenues	<u>35,100</u>	<u>18,096</u>	<u>108,085</u>	<u>40,893</u>
Operating expenses:				
Cost of sales	1,052	1,394	2,705	2,407
Research and development	37,185	31,579	150,813	122,340
Selling, general and administrative	33,929	28,389	126,417	105,421
Total operating expenses	<u>72,166</u>	<u>61,362</u>	<u>279,935</u>	<u>230,168</u>
Loss from operations	<u>(37,066)</u>	<u>(43,266)</u>	<u>(171,850)</u>	<u>(189,275)</u>
Other income (expense):				
Interest income	396	1,102	2,820	5,422
Interest expense	(7,072)	(6,467)	(27,140)	(15,647)
Other income (expense), net	383	(14)	206	(50)
Total other expense, net	<u>(6,293)</u>	<u>(5,379)</u>	<u>(24,114)</u>	<u>(10,275)</u>
Loss before income taxes	<u>(43,359)</u>	<u>(48,645)</u>	<u>(195,964)</u>	<u>(199,550)</u>
Income tax provision	(62)	(2)	(309)	(40)
Net loss	<u>\$ (43,421)</u>	<u>\$ (48,647)</u>	<u>\$ (196,273)</u>	<u>\$ (199,590)</u>
Net loss per share—basic and diluted	<u>\$ (0.59)</u>	<u>\$ (0.76)</u>	<u>\$ (2.72)</u>	<u>\$ (3.22)</u>
Weighted-average number of common shares outstanding used in net loss per share—basic and diluted	<u>73,727</u>	<u>63,908</u>	<u>72,044</u>	<u>61,955</u>

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

	<u>December 31, 2020</u>	<u>December 31, 2019</u>
<b>Assets</b>		
Cash, cash equivalents and investments	\$ 273,455	\$ 263,972
Restricted cash	3,203	1,831
Accounts receivable	12,881	7,862
Property and equipment, net	2,219	3,046
Other assets	21,292	18,252
Total assets	<u>\$ 313,050</u>	<u>\$ 294,963</u>
<b>Liabilities and stockholders' equity</b>		
Deferred revenue	\$ 297	\$ 4,533
Convertible senior notes	117,928	109,857
Deferred royalty obligation	73,088	73,588
Other liabilities	71,191	57,211
Total liabilities	<u>262,504</u>	<u>245,189</u>
Total stockholders' equity	<u>50,546</u>	<u>49,774</u>
Total liabilities and stockholders' equity; 73,923 and 65,370 shares issued and outstanding at December 31, 2020 and December 31, 2019, respectively	<u>\$ 313,050</u>	<u>\$ 294,963</u>

SOURCE Karyopharm Therapeutics Inc.

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<https://investors.karyopharm.com/2021-02-11-Karyopharm-Reports-Fourth-Quarter-and-Full-Year-2020-Financial-Results-and-Highlights-Recent-Company-Progress>