

# Karyopharm Reports Fourth Quarter and Full Year 2019 Financial Results and Highlights Recent Company Progress

- XPOVIO Net Product Sales of \$17.7 Million for Fourth Quarter and \$30.5 Million for Full Year 2019 --
- XPOVIO Prescription Demand Increased by 65% in Fourth Quarter 2019 Compared to Third Quarter 2019 --
- sNDA for Selinexor in DLBCL Submitted to FDA --
- Phase 3 BOSTON Top-Line Data Expected Before End of April 2020 --
- Conference Call Scheduled for Today at 8:30 a.m. ET --

NEWTON, Mass., Feb. 13, 2020 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), an oncology-focused pharmaceutical company, today reported financial results for the fourth quarter and full year ended December 31, 2019. In addition, Karyopharm highlighted select corporate milestones, including details regarding the ongoing U.S. commercial launch of XPOVIO® (selinexor), and provided an overview of its key clinical development programs.

“2019 was a year of significant progress for Karyopharm as we successfully transitioned into an integrated commercial organization following the accelerated approval of oral XPOVIO, the first and only nuclear export inhibitor approved in the U.S., indicated for patients with heavily pretreated multiple myeloma. Our XPOVIO commercial launch efforts have yielded a strong positive reception from prescribing physicians and patients, with approximately 1,400 prescriptions filled in 2019,” said Michael G. Kauffman, MD, PhD, Chief Executive Officer of Karyopharm. “Our pipeline efforts also continued to advance with the top-line Phase 3 BOSTON study results expected before the end of April, which if positive, could support future regulatory submissions and dramatically increase the eligible multiple myeloma patient population for XPOVIO. And importantly, we ended the year with the submission of our supplemental New Drug Application (sNDA) in the U.S. based on our SADAL study for selinexor requesting accelerated approval for patients with relapsed or refractory diffuse large B-cell lymphoma (DLBCL), a disease with significant patient unmet need.”

## Fourth Quarter 2019 Highlights and Recent Progress

### XPOVIO (selinexor) in Multiple Myeloma

- XPOVIO U.S. Commercial Launch Continues Momentum. Oral XPOVIO became commercially available to patients in the U.S on July 9, 2019 and generated net product sales of \$17.7 million in the fourth quarter and \$30.5 million for the full year 2019. As of December 31, 2019, approximately 1,400 XPOVIO prescriptions have been filled, driven by strong demand from both academic and community-based oncologists. XPOVIO has been prescribed by more than 550 unique physicians and healthcare accounts. Net product sales increased by approximately 38% in the fourth quarter of 2019 as compared to the third quarter of 2019 with prescription demand (defined as prescriptions shipped from Karyopharm’s distribution partners to individual patients and prescribing healthcare accounts) increasing by approximately 65% in the fourth quarter as compared to the third quarter of 2019.

Increased fourth quarter net revenues were primarily driven by a combination of new patient starts and prescription refills. Broad insurance coverage for XPOVIO has been a key contributor to its commercial success with XPOVIO being added to numerous national commercial and Medicare formularies and coverage policies. Based on prescription fulfillment data through the specialty pharmacy channel, Karyopharm estimates that approximately 55% of XPOVIO prescriptions have been dispensed to patients with Medicare coverage, 40% to patients with commercial insurance, and the remaining patients having either Medicaid or another form of prescription coverage.

- 22 Abstracts Presented at the American Society of Hematology (ASH) 2019 Annual Meeting. Multiple data presentations highlighting selinexor at the ASH 2019 Annual Meeting held December 7-10, 2019, continue to reinforce the potential clinical utility of selinexor as a new therapeutic option for patients with relapsed or refractory multiple myeloma. This included one oral presentation which provided updated data from the Phase 1b/2 STOMP study evaluating the all oral regimen of selinexor in combination with Pomalyst® (pomalidomide) and low-dose dexamethasone (dex) (SPd) in patients with relapsed or refractory multiple myeloma. Patients with Pomalyst-naïve and Revlimid® (lenalidomide)-relapsed or -refractory myeloma achieved a 56% overall response rate (ORR) and a 12.2-month progression free survival (PFS) on the SPd regimen. A separate poster presentation detailed results for the Kyprolis® (carfilzomib) arm of the Phase 1b/2 STOMP study which showed a 71% ORR and a 21% complete response rate (CR) in patients with heavily pretreated Kyprolis-naïve multiple myeloma. New data from patients treated with selinexor-based regimens after their myeloma had progressed following experimental CAR-T (chimeric antigen receptor modified T cell) therapy was also highlighted in a poster and demonstrated promising early responses from six of seven patients, reinforcing the potential therapeutic activity of selinexor in patients with advanced refractory disease.
- Decision from European Medicines Agency (EMA) for Marketing Authorization Application (MAA) Now Expected in Mid-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 to provide additional time to respond to the outstanding questions related to the MAA. The Company now expects a decision on the selinexor MAA in mid-2020.
- Pivotal Phase 3 BOSTON Study Remains On Track. Top-line data are expected before the end of April 2020 contingent upon the occurrence of PFS events, the primary endpoint of the study. The BOSTON study is evaluating 100mg of selinexor dosed *once* weekly in combination with the proteasome inhibitor Velcade® (bortezomib) (*once* weekly) and low dose dexamethasone (SVd), compared to standard *twice* weekly Velcade and low dose dexamethasone (Vd) in patients with multiple myeloma who have had one to three prior lines of therapy. Data from the BOSTON study, if positive, are expected to be used to support regulatory submissions to the U.S. Food and Drug Administration (FDA) and EMA requesting the use of selinexor in combination with Velcade and dexamethasone in patients with multiple myeloma who have received at least one prior therapy.

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

- sNDA Submitted in December 2019. Following the positive results from the Phase 2b SADAL study that were first presented at the ASH 2018 Annual Meeting and then updated in June 2019 at the 2019 International Conference on Malignant Lymphoma, Karyopharm submitted a sNDA to the FDA in December 2019 requesting accelerated approval for selinexor as a treatment for patients with relapsed or refractory DLBCL after at least two prior multi-agent therapies and who are ineligible for stem cell transplantation including CAR-T therapy. The Company also expects to submit an MAA to the EMA in 2020 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.

### Selinexor in Solid Tumors

- Ongoing Phase 3 Portion of the Phase 2/3 SEAL Study in Liposarcoma. Karyopharm previously reported positive results from the Phase 2 portion of the randomized, blinded Phase 2/3 SEAL study evaluating single-agent selinexor versus placebo in patients with previously treated, advanced unresectable dedifferentiated liposarcoma. Enrollment is currently ongoing in the Phase 3 portion of the SEAL study. Top-line data from the Phase 3 portion of the SEAL study are anticipated in 2020. Assuming a positive outcome on the primary endpoint of PFS, the Company intends to use the data from the SEAL study to support NDA and MAA submissions requesting approval for selinexor for patients with advanced unresectable dedifferentiated liposarcoma.

### Other Pipeline Updates

- Five New Clinical Trials Expected to Start in 2020 to Investigate Karyopharm Drug Candidates Across Both Hematologic Malignancies and Solid Tumors:
  - DLBCL: XPORT-DLBCL-030 is a Phase 2/3 trial expected to serve as a confirmatory study for the accelerated approval requested in DLBCL based on the SADAL study. This trial will study selinexor or a matching placebo given with the standard combination immunotherapy R-GDP (rituximab, gemcitabine, dexamethasone, cisplatin) to patients with at least one prior therapy and ineligible for high dose chemotherapy and stem cell transplantation (or CAR-T). The primary endpoint of the study is PFS.
  - DLBCL: XPORT-DLBCL-025 is a multi-arm Phase 1/2 trial of selinexor in combination with commonly used and approved agents for the treatment of DLBCL. This study will inform the clinical development of selinexor with a variety of additional agents for the treatment of DLBCL.
  - Colorectal Cancer and Lung Cancer: XPORT-STP-027 is a Phase 1/2 trial designed to study selinexor in combination with pembrolizumab in colorectal cancer and separately in combination with docetaxel in non-small cell lung cancer.
  - Glioblastoma (GBM): XPORT-GBM-029 is a Phase 1/2 trial designed to study selinexor in combination with active anti-cancer agents in both newly diagnosed and patients with recurrent disease.
  - Myelodysplastic Syndromes (MDS): Based on positive data from an ongoing Phase 1 / 2 trial of single-agent eltanexor presented at ASH 2019, we plan to evaluate the combination of eltanexor with cedazuridine-decitabine (ASTX727) in patients with newly diagnosed MDS.
- Encouraging Pre-Clinical Data Highlighting Potential Role of KPT-9274 Published in *Nature Cancer*: In January 2020, results from a study conducted by researchers at the University of California, Los Angeles (UCLA) Jonsson Comprehensive Cancer Center were published in *Nature Cancer*. Researchers concluded that inhibiting the kinase PAK4 improves the effectiveness of PD-1 blockade immunotherapy in melanoma cells. KPT-9274, Karyopharm's investigational dual PAK4 and NAMPT inhibitor, was given in an animal model of melanoma in combination with anti-PD-1 immunotherapy, effectively slowing the growth of melanomas more than either drug alone. Finding novel molecular targets that could improve and overcome resistance to PD-1 blockade therapy remains a key priority in current cancer research and the results from this study support future clinical development of KPT-9274 in combination with anti-PD-1 therapy.

#### Corporate and Financial Updates

- Executed Licensing Agreement with Neopharm Group to Commercialize XPOVIO in Israel. In February 2020, Karyopharm and Promedico, a fully-owned Neopharm LTD company, entered into an exclusive distribution agreement for the commercialization of XPOVIO in Israel and the Palestinian Authority. Karyopharm will receive certain prespecified payments and is eligible to receive additional payments if prespecified regulatory and commercial milestones are achieved by Promedico. Karyopharm is also eligible to receive double-digit royalties on future net sales in the territory. Promedico is responsible for all regulatory filings and obligations required for registering XPOVIO. Karyopharm has retained exclusive production rights and will supply finished product for commercial use in the territory.

#### Full Year and Fourth Quarter 2019 Financial Results

Net product revenue: Net product revenue for the fourth quarter of 2019 was \$17.7 million and \$30.5 million for the year ended December 31, 2019. Karyopharm did not have net product revenue during the year ended December 31, 2018.

License and other revenue: License and other revenue for the fourth quarter of 2019 was \$0.4 million, compared to \$0.2 million for the fourth quarter of 2018. License and other revenue for the year ended 2019 was \$10.4 million, compared to \$30.3 million for the year ended 2018.

Cost of sales: Karyopharm began U.S. sales of XPOVIO in the third quarter of 2019. Cost of sales were \$1.4 million for the fourth quarter of 2019, and \$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 expenses: Research and development expense for the fourth quarter of 2019 was \$31.6 million, compared to \$38.9 million for the fourth quarter of 2018. Research and development expense for the year ended 2019 was \$122.3 million, compared to \$161.4 million for the year ended 2018.

Selling, general and administrative expenses: For the fourth quarter 2019, selling, general and administrative expense was \$28.4 million, compared to \$18.8 million for the fourth quarter 2018. For the year ended December 31, 2019, selling, general and administrative expense was \$105.4 million, compared to \$48.8 million for the year ended December 31, 2018. The increase in selling, general and administrative expenses compared to the prior year was due primarily to the hiring of the Karyopharm commercial team and related activities to support the U.S. commercial launch of XPOVIO.

Interest expense: Interest expense for the fourth quarter and for the year ended December 31, 2019 was \$6.5 million and \$15.6 million, respectively, compared to \$2.5 million for both the fourth quarter 2018 and the year ended December 31, 2018. The increase in interest expense is attributable to the imputed interest on the deferred royalty obligation Karyopharm has with HealthCare Royalty Partners (HCR).

Net loss: Karyopharm reported a net loss of \$48.6 million, or \$0.76 per share, for the fourth quarter 2019, compared to a net loss of \$58.2 million, or \$0.96 per share, for the fourth quarter 2018. Net loss includes non-cash stock-based compensation expense of \$3.6 million and \$3.9 million for the 2019 and 2018 quarters, respectively. Karyopharm reported a net loss of \$199.6 million, or \$3.22 per share, for the year ended 2019, compared to a net loss of \$178.4 million, or \$3.14 per share, for the year ended 2018. Net loss includes non-cash stock-based compensation expense of \$15.3 million and \$17.3 million for the years ended 2019 and 2018, respectively.

Cash position: Cash, cash equivalents, restricted cash and investments as of December 31, 2019 totaled \$265.8 million, compared to \$330.9 million as of December 31, 2018.

#### 2020 Financial Outlook

Based on its current operating plans, Karyopharm expects 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 million to \$260 million.

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 2021.

#### Non-GAAP Financial Information

Karyopharm uses a non-GAAP financial measure, non-GAAP operating expense, to provide operating expense guidance. Non-GAAP operating expense excludes 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. Karyopharm has not reconciled the full year 2020 outlook for non-GAAP operating expenses to full year 2020 outlook for GAAP operating 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 operating expenses. This non-GAAP financial measure should not be considered a substitute or an alternative to GAAP total operating expense and should not be considered a measure of Karyopharm's liquidity. Instead, non-GAAP operating expense should only be used to supplement an understanding of Karyopharm's operating results as reported under GAAP.

## Conference Call Information

Karyopharm will host a conference call today, Thursday, February 13, 2020, at 8:30 a.m. Eastern Time, to discuss the fourth quarter and full year 2019 financial results, recent accomplishments, clinical developments and business plans. To access the conference call, please dial (855) 437-4406 (local) or (484) 756-4292 (international) at least 10 minutes prior to the start time and refer to conference ID 4367549. 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 ≥3 infections were reported in 25% of patients, and deaths resulting from an infection occurred in 4% of patients. The most commonly reported Grade ≥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 oncology-focused pharmaceutical company dedicated to the discovery, development, and commercialization of novel first-in-class drugs directed against nuclear export and related targets for the treatment of cancer and other major diseases. Karyopharm's 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. A Marketing Authorization Application for selinexor is also currently under review by the European Medicines Agency. Karyopharm recently submitted a New Drug Application to the FDA seeking accelerated approval for XPOVIO in patients with diffuse large B-Cell lymphoma. 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 relating to 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; and the therapeutic potential of and potential clinical development plans for Karyopharm's drug candidates, especially selinexor. Such statements are subject to numerous important factors, risks and uncertainties, many of which are beyond Karyopharm's control, that may cause actual events or results to differ materially from Karyopharm's current expectations. For example, there can be no guarantee that Karyopharm will successfully commercialize XPOVIO; that regulators will agree that selinexor qualifies for conditional approval in the E.U. as a result of data from the STORM study or 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: 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 September 30, 2019, which was filed with the Securities and Exchange Commission (SEC) on November 4, 2019, and in other filings that Karyopharm may make with the SEC in the future. Any forward-looking statements contained in this press release speak only as of the date hereof, and, except as required by law, Karyopharm expressly disclaims any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise.

Velcade® is a registered trademark of Takeda Pharmaceutical Company Limited

Pomalyst® is a registered trademark of Celgene Corporation

Kyprolis® is a registered trademark of Onyx Pharmaceuticals, Inc.

#### 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)

(unaudited)

	Three Months Ended, December 31,		Twelve Months Ended December 31,	
	2019	2018	2019	2018
Revenues:				
Product revenue, net	\$ 17,719	\$ —	\$ 30,540	\$ —
License and other revenue	377	206	10,353	30,336
Total revenues	18,096	206	40,893	30,336
Operating expenses:				
Cost of sales	1,394	—	2,407	—
Research and development	31,579	38,890	122,340	161,372
Selling, general and administrative	28,389	18,771	105,421	48,847
Total operating expenses	61,362	57,661	230,168	210,219
Loss from operations	(43,266 )	(57,455 )	(189,275 )	(179,883 )
Other income (expense):				
Interest income	1,102	1,768	5,422	4,028
Interest expense	(6,467 )	(2,493 )	(15,647 )	(2,493 )
Other expense	(14 )	(13 )	(50 )	(33 )
Total other (expense) income, net	(5,379 )	(738 )	(10,275 )	1,502
Loss before income taxes	(48,645 )	(58,193 )	(199,550 )	(178,381 )
Income tax provision	(2 )	(17 )	(40 )	(26 )
Net loss	\$ (48,647 )	\$ (58,210 )	\$ (199,590 )	\$ (178,407 )
Net loss per share—basic and diluted	\$ (0.76 )	\$ (0.96 )	\$ (3.22 )	\$ (3.14 )
Weighted-average number of common shares outstanding used in net loss per share—basic and diluted	63,908,471	60,759,500	61,955,420	56,799,699

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

	December 31, 2019	December 31, 2018
ASSETS		
Cash, cash equivalents and investments	\$ 263,972	\$ 330,200
Restricted cash	1,831	716
Accounts receivable	7,862	—
Property and equipment, net	3,046	3,863
Other assets	18,252	6,413
Total assets	\$ 294,963	\$ 341,192
LIABILITIES AND STOCKHOLDERS' EQUITY		
Deferred revenue	4,533	13,894
Convertible senior notes	109,857	102,664
Deferred royalty obligation	73,588	—
Other liabilities	57,211	41,464
Total stockholders' equity	49,774	183,170
Total liabilities and stockholders' equity	\$ 294,963	\$ 341,192

Source: Karyopharm Therapeutics Inc.



