



March 16, 2017

Karyopharm Reports Fourth Quarter and Full Year 2016 Financial Results and Provides Selinexor Clinical Update

– Overall Response Rate of 28.4% Observed in Phase 2b SADAL Study in Relapsed/Refractory DLBCL; Additional Top-line Data to be Presented as a Late-Breaker at AACR 2017 Annual Meeting —

– Company Announces FDA-Affirmed Development Path for Selinexor in DLBCL with Potential for Accelerated Approval —

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

NEWTON, Mass., March 16, 2017 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI), a clinical-stage pharmaceutical company, today reported financial results for the fourth quarter and full year 2016 and provided a clinical update for selinexor (KPT-330), its lead, novel, oral Selective Inhibitor of Nuclear Export (SINE™) compound. The Company also provided an overview of select accomplishments related to its other pipeline assets, including KPT-8602, its second-generation oral SINE™ compound, KPT-9274, its oral, dual inhibitor of p21-activated kinase 4 (PAK4) and nicotinamide phosphoribosyltransferase (NAMPT), and KPT-350, an oral SINE™ compound with potential applications in neurological and autoimmune indications.

"2016 marked a year of several key achievements for Karyopharm, including the rapid advancement of oral selinexor in relapsed or refractory multiple myeloma (MM), a disease for which we believe we have a path to regulatory approval," said Michael G. Kauffman, MD, PhD, Chief Executive Officer of Karyopharm. "To date, 2017 has brought several significant developments. Today we announced the planned development path for selinexor in diffuse large B-cell lymphoma (DLBCL), our second lead indication after MM. Following the observation of a 28.4% overall response rate (ORR) in the Phase 2b SADAL study, we consulted with the U.S. Food and Drug Administration (FDA) and obtained their agreement to our amendment of the SADAL study to focus solely on the 60mg twice weekly treatment cohort, in which we plan to enroll up to 90 more patients. Assuming we continue to see the response rate we have observed to date, we intend to use the data from the SADAL study to support an accelerated approval in DLBCL. We look forward to presenting additional top-line data from the Phase 2b SADAL study in a late-breaking poster at the upcoming American Association for Cancer Research (AACR) 2017 annual meeting in early April."

As previously announced on March 10, 2017, Karyopharm's selinexor clinical trials have been placed on a partial clinical hold by the FDA due to incomplete information in the existing version of the investigator's brochure (IB), including an incomplete list of serious adverse events (SAEs) associated with selinexor. The partial clinical hold is not the result of any patient death or any new information regarding the safety profile of selinexor. While the partial clinical hold remains in effect, patients with stable disease or better may remain on selinexor therapy, but no new patients may be enrolled until the partial clinical hold is lifted. The Company has provided all requested materials to the FDA believed to be required to lift the partial clinical hold. Karyopharm is working diligently with the FDA to seek the release of the partial clinical hold and resume enrollment in its selinexor clinical trials as expeditiously as possible. The Company believes that its previously disclosed enrollment rates and timelines for its ongoing trials will remain materially unchanged.

Clinical Update for Selinexor in DLBCL:

Today Karyopharm reported a 28.4% overall response rate (ORR) in the ongoing Phase 2b SADAL study evaluating 60mg and 100mg of selinexor dosed twice weekly in patients with relapsed or refractory DLBCL. In a recent analysis of the first 63 patients between both arms, the ORR, as determined by independent Central Radiological Review, was 28.4%, with consistent response rates across both arms, but greater durability and tolerability observed in the 60mg arm. As a result of these findings, and in consultation with the FDA, the Company has decided to amend the SADAL study protocol to become a single-arm study focusing solely on single-agent selinexor dosed at 60mg twice weekly, eliminating the 100mg arm. The Company will also make certain other protocol amendments, including reducing the 14-week washout period to 8 weeks for patients who achieved at least a partial response on their most recent therapy. The FDA agreed that the changes to the single-arm study were reasonable and that the proposed trial design and indication appear appropriate for accelerated approval, though eligibility for accelerated approval will depend on the complete trial results and available therapies at the time of regulatory action. The Company plans to enroll up to an additional 90 patients to the new 60mg single-arm cohort, and expects to report top-line results from the completed SADAL study in mid-2018.

Dr. Kauffman continued, "Looking ahead to the remainder of 2017, we are focusing on initiation of the pivotal Phase 3 BOSTON trial where we will evaluate selinexor in combination with Velcade® (bortezomib) and dexamethasone in patients with MM previously treated with one to three regimens, moving selinexor into much earlier lines of treatment. After the presentation of the SADAL data at the AACR 2017 annual meeting, we anticipate multiple other important data readouts during the year, including top-line data from the Phase 2 portion of our randomized Phase 2/3 SEAL study in patients with liposarcoma, our most advanced solid tumor indication in mid-2017. We also plan to present top-line Phase 1 safety and tolerability data for KPT-9274 in the second half of the year."

"Our other key objectives include continued execution of the expanded STORM and STOMP studies in relapsed or refractory MM. For STORM, the expansion arm evaluating oral selinexor in patients with penta-refractory MM is expected to read out in early 2018. Assuming a positive outcome, we intend to use the STORM study data to support accelerated approval for selinexor in MM. For STOMP, we recently completed enrollment in the selinexor, Velcade® (bortezomib) and dexamethasone arm and expect to soon initiate a new expansion arm evaluating oral selinexor in combination with the anti-CD38 monoclonal antibody Darzalex®," Dr. Kauffman concluded.

Fourth Quarter 2016 and Recent Events, Highlights and Milestones:

Selinexor in Multiple Myeloma (MM)

- 1 **Initiating Pivotal Phase 3 BOSTON Study in Early 2017.** Based on the strong combination data recently reported from the Phase 1b STOMP study, Karyopharm plans to initiate a pivotal randomized Phase 3 study, known as the BOSTON (**B**ortezomib, **S**elinexor and **d**examethasone) study, which will evaluate selinexor in combination with Velcade® (bortezomib) and dexamethasone (SVd), compared to Velcade® and low-dose dexamethasone (Vd) in patients with MM who have had one to three prior lines of therapy. Karyopharm has identified the combination dose of selinexor (100mg weekly), Velcade® (1.3 mg/m² weekly given subcutaneously for 4 of 5 weeks) and dexamethasone (40mg weekly) to be used in the BOSTON study and expects that the study will enroll approximately 360 patients. The Company expects to commence the BOSTON study in early 2017.
- 1 **Expanded STORM Study to Include 122 Additional Patients with Penta-refractory MM.** The Company has expanded the STORM study, which is expected to include 122 additional patients with penta-refractory MM, a growing unmet medical need in which there are no approved therapies available. Karyopharm expects to report top-line data from the expanded cohort in early 2018, and, assuming a positive outcome, intends to use the expanded STORM study data to support accelerated approval for selinexor in MM.
- 1 **Completed Enrollment in STOMP Arm Evaluating Selinexor in Combination with Velcade.** In February 2017, Karyopharm completed enrollment in the Phase 1b/2 STOMP arm designed to evaluate selinexor in combination with the proteasome inhibitor Velcade® and low-dose dexamethasone (SVd) in heavily pretreated patients with MM. The SVd arm of the STOMP study enrolled 42 patients and the Company expects to report updated data towards the end of 2017.
- 1 **On Track to Initiate New STOMP Expansion Arm Evaluating Selinexor in Combination with Darzalex® (daratumumab).** Karyopharm expects to dose the first patient in a new Phase 1b/2 STOMP expansion arm designed to evaluate selinexor in combination with the anti-CD38 monoclonal antibody Darzalex® and low-dose dexamethasone (SDd) in heavily pretreated patients with MM. The SDd arm of the STOMP study is expected to enroll approximately 44 patients and the Company expects to report top-line data in late 2017 or early 2018.
- 1 **Reported Updated STORM and STOMP Data at ASH 2016 Annual Meeting.** Karyopharm presented updated clinical data from the ongoing Phase 2b STORM study and the ongoing Phase 1b STOMP study at the American Society of Hematology (ASH) 2016 annual meeting. The updated STORM data demonstrated that patients treated with selinexor plus low-dose dexamethasone achieved an overall response rate (ORR), adjudicated by an Independent Review Committee, of 21% (n=78), and that the ORR was similar between patients with quad-refractory (21%; n=48) and penta-refractory (20%; n=30) disease. The updated STOMP data showed that selinexor in combination with Velcade® (bortezomib) and dexamethasone (SVd) produced an ORR of 77% (investigator assessed) across all evaluable patients in the study (n=22), including patients with MM not refractory to a proteasome inhibitor (ORR 100%; n=7) and those with disease previously refractory to a proteasome inhibitor (ORR 67%; n=15).
- 1 **Co-hosted Expert Panel Discussion with the Multiple Myeloma Research Foundation (MMRF) at ASH 2016 Annual Meeting.** Karyopharm and the MMRF hosted a panel discussion featuring leading MM thought leaders at the ASH 2016 annual meeting. Topics discussed by recognized expert panelists included the need for new MM treatments with novel mechanisms of action and the combinability of MM agents for synergistic activity. A webcast replay and transcript of the panel discussion are available at <http://investors.karyopharm.com/events.cfm>.
- 1 **Presented an Overview of Clinical Data Demonstrating Selinexor Activity in Combination with Proteasome Inhibitors and Immunomodulatory Agents.** In an oral presentation at the International Myeloma Workshop 2017

annual meeting held March 1-4, 2017 in New Delhi, India, Karyopharm researchers presented an overview of clinical data demonstrating selinexor's activity in combination with proteasome inhibitors and immunomodulatory drugs for the treatment of relapsed or refractory MM.

Selinexor in Diffuse Large B-Cell Lymphoma

- 1 **Top-line Data from Phase 2b SADAL Study in DLBCL Selected as a Late-Breaking Abstract at AACR 2017 Annual Meeting.** In March 2017, Karyopharm announced that an abstract highlighting top-line data from its Phase 2b SADAL study evaluating single-agent selinexor with dexamethasone in patients with relapsed or refractory DLBCL was selected as a late-breaking poster at the AACR 2017 Annual Meeting. The poster will be presented by Marie Maerevoet, Institute Jules Bordet, Belgium, on Tuesday, April 4, 2017 from 1:00-5:00PM ET.

Selinexor in Other Hematologic Malignancies

- 1 **Announced Outcome of Phase 2 SOPRA Interim Analysis; Updated AML Development Strategy.** In March 2017, Karyopharm announced the results of the planned interim analysis of the Phase 2 SOPRA study evaluating single-agent selinexor in relapsed or refractory acute myeloid leukemia (AML). In concert with the study's independent Data Safety Monitoring Board (DSMB), the Company determined that the SOPRA study would not reach statistical significance for showing superiority of overall survival (OS) on selinexor versus OS on physician's choice (PC), the study's primary endpoint. However, since the 13% of selinexor-treated patients who achieved a complete response with or without full hematologic recovery (CR/CRi) showed a substantial OS benefit as compared to PC, they will be permitted to continue on the selinexor arm or the PC arm, as applicable, following discussion between the patient and his or her treating physician. Importantly, selinexor demonstrated a safety profile consistent with previous studies. Importantly, in the selinexor arm, there were similar rates of sepsis and lower rates of febrile neutropenia compared with the PC arm. Karyopharm plans to continue to explore the use of selinexor in combination with novel and standard agents through investigator-sponsored AML studies in both adults and children.
- 1 **Reported Final Data from Phase 2 SAIL Study in AML at ASH 2016 Annual Meeting.** Final clinical data from the Phase 2 SAIL study evaluating selinexor in combination with Ara-C and idarubicin demonstrated a compelling 55% ORR in heavily pretreated patients with relapsed or refractory AML. We believe that selinexor in combination with Ara-C and idarubicin may be an effective AML treatment option and serve as a bridge to stem cell transplantation in this patient population. Given the encouraging data observed to date across these settings, Karyopharm plans to continue clinical development of selinexor in AML through investigator sponsored trials in multiple combination regimens, including with chemotherapy.
- 1 **Published Clinical Data Demonstrating Selinexor's Activity in Pediatric Patients with Relapsed/Refractory Leukemia in the *Journal of Clinical Oncology*.** A paper describing results from the investigator-sponsored SELHEM study evaluating selinexor's activity in pediatric patients with relapsed or refractory leukemia were recently published in the *Journal of Clinical Oncology*. In the paper, authored by Thomas B. Alexander, et al., titled "[Phase I Study of Selinexor, a Selective Inhibitor of Nuclear Export, in Combination With Fludarabine and Cytarabine, in Pediatric Relapsed or Refractory Acute Leukemia](#)," Karyopharm collaborators report that seven of the 15 evaluable patients, or 47%, achieved either a CR or a CRi. Five of the responses included CRs negative for minimal residual disease (MRD) and two patients had MRD negative CRs within the first cycle after receiving only selinexor therapy prior to any chemotherapy. Based on these data, Karyopharm plans to explore the benefit of selinexor in combination with intensive chemotherapy through investigator-sponsored Phase 2 clinical trials in pediatric patients with heavily pretreated AML.

Selinexor in Solid Tumors

- 1 **Completed Enrollment in Phase 2 Portion of the SEAL Study.** In March 2017, Karyopharm completed enrollment in the randomized Phase 2 portion of the SEAL study evaluating selinexor in patients with advanced liposarcoma. The company expects to report top-line data in mid-2017.
- 1 **Reported Final Data from Phase 2 SIGN Study in Gynecologic Malignancies.** In October 2016, Karyopharm reported final data from its Phase 2 SIGN study evaluating oral selinexor for the treatment of gynecological malignancies, including ovarian, endometrial and cervical cancers, at the European Society of Medical Oncology 2016 annual meeting. Of the 20 evaluable patients with endometrial cancer, 9 met the primary endpoint (3 confirmed partial responses and 6 with stable disease of ≥ 12 weeks), for a disease control rate of 45%. Median progression-free survival for the endometrial cancer arm was 3 months and median OS was 8 months. An investigator-sponsored Phase 3 randomized double-blind study evaluating selinexor in patients with advanced or recurrent endometrial cancer is in development and expected to initiate enrollment in late 2017.

Selinexor Early Scientific Research

- | **Published Preclinical Data Demonstrating Selinexor Anti-Tumor Activity in Combination with Immunotherapeutic Agents.** Two papers describing the synergistic anti-tumor activity of selinexor in combination with immunotherapeutic agents, including PD-1 or PD-L1 checkpoint inhibitors, and further validating the selinexor clinical dosing schedules, were published online in *Molecular Cancer Therapeutics*. In the first paper, authored by Matthew R. Farren et al., titled "[The exportin-1 inhibitor selinexor exerts superior anti-tumor activity when combined with T cell checkpoint inhibitors](#)," Karyopharm researchers, in collaboration with Emory University and Ohio State University, report that selinexor combined with immune checkpoint inhibitors, including PD-1, PD-L1 or CTLA-4 blocking antibodies, significantly limited tumor growth in an aggressive murine model of melanoma. The reduction in tumor growth was accompanied by systemic changes in natural killer cells, myeloid derived suppressor cells, T cell differentiation and increased infiltration of T cells in the tumor microenvironment.
- | In the second paper, authored by Paul M. Tyler et al., titled "[Clinical dosing regimen of selinexor maintains normal immune homeostasis and T cell effector function in mice: implications for combination with immunotherapy](#)," Karyopharm researchers, in collaboration with the Dana-Farber Cancer Institute, University of Amsterdam and Massachusetts General Hospital, discuss preclinical results supporting further evaluation of selinexor in combination with anti-PD-1 monoclonal antibodies as a potential treatment approach for cancer patients. In this study, it was determined that selinexor in combination with anti-PD-1 monoclonal antibodies, dosed twice weekly is the optimal dosing schedule to allow sufficient time for a fully functional CD8 T cell response and development of anti-tumor immunity. Therefore, the combination of selinexor with a PD-1 or PD-L1 checkpoint inhibitor was predicted to have added benefit over selinexor treatment alone.

KPT-8602

- | **Reported Phase 1 Clinical Data for KPT-8602 at ASH 2016 Annual Meeting.** Clinical data from a Phase 1/2 study evaluating KPT-8602, Karyopharm's second-generation SINE™ compound, were presented at the ASH 2016 annual meeting by Frank Cornell, MD, Vanderbilt Ingram Cancer Center. These data demonstrated that oral KPT-8602 was well tolerated in heavily pretreated patients with relapsed or refractory MM and showed early signs of encouraging efficacy.

KPT-9274

- | **Preclinical Data Highlighting KPT-9274's Anti-Cancer Activity in Dogs Selected as a Late-Breaking Abstract at AACR 2017 Annual Meeting.** In March 2017, Karyopharm announced that an abstract highlighting preclinical data demonstrating KPT-9274's activity and synergy with doxorubicin to treat dogs with lymphoma was selected as a late-breaking poster at the AACR 2017 annual meeting. The poster will be presented by Cheryl London, Tufts University, on Wednesday, April 5, 2017 from 8:00 AM-12:00PM ET.

KPT-350

- | **Target ALS Consortium Grants \$900,000 in Research Funding.** The Target ALS Foundation awarded a \$900,000 grant to support preclinical studies of KPT-350 in amyotrophic lateral sclerosis (ALS). The project, led by Karyopharm in collaboration with researchers from Johns Hopkins University and the University of Florida, is studying KPT-350 in preclinical models and will seek to develop an oral suspension formulation to dose patients who cannot swallow tablets.

Fourth Quarter and Year Ended December 31, 2016 Full Year Financial Results

Cash, cash equivalents and investments as of December 31, 2016, including restricted cash, totaled \$175.5 million, compared to \$210.0 million as of December 31, 2015.

For the year ended December 31, 2016, research and development expense was \$86.9 million compared to \$97.7 million for the year ended December 31, 2015. For the year ended December 31, 2016, general and administrative expense was \$23.9 million compared to \$21.6 million for the year ended December 31, 2015.

Karyopharm reported a net loss of \$109.6 million, or \$2.92 per share, for the year ended December 31, 2016, compared to a net loss of \$118.2 million, or \$3.32 per share, for the year ended December 31, 2015. Net loss includes stock-based compensation expense of \$22.3 million and \$17.1 million for the years ended December 31, 2016 and December 31, 2015, respectively.

For the quarter ended December 31, 2016, research and development expense was \$20.7 million compared to \$24.1 million for the quarter ended December 31, 2015. The decrease in research and development expenses resulted primarily from the timing of clinical expenses related to the development of selinexor. For the quarter ended December 31, 2016, general

and administrative expense was \$6.5 million compared to \$5.3 million for the quarter ended December 31, 2015. Karyopharm reported a net loss of \$26.9 million, or \$0.65 per share, for the quarter ended December 31, 2016, compared to a net loss of \$29.0 million, or \$0.81 per share, for the quarter ended December 31, 2015. Net loss includes stock-based compensation expense of \$5.1 million and \$5.4 million for the quarters ended December 31, 2016 and December 31, 2015, respectively.

Financial Outlook

Karyopharm expects its operating cash burn, including research and development and general and administrative expenses, for the year ending December 31, 2017 to be in the range of \$85 to 90 million. Based on current operating plans, Karyopharm expects that its existing cash and cash equivalents will fund its research and development programs and operations until the end of 2018, including through the data readout for the expanded STORM cohort, completion of enrollment for the BOSTON study and the advancement of other ongoing selinexor clinical studies to their next data inflection points.

Conference Call Information:

Karyopharm will host a conference call today, Thursday, March 16, 2017, at 8:30 a.m. Eastern Time, to discuss the fourth quarter and full-year 2016 financial results, recent accomplishments, clinical developments and business plans. To access the conference call, please dial (855) 437-4406 (US) or (484) 756-4292 (international) at least five minutes prior to the start time and refer to conference ID: 79794446. An audio recording of the call will be available under "Events & Presentations" in the "Investor" section of Karyopharm's website, <http://www.karyopharm.com>, approximately two hours after the event.

About Karyopharm Therapeutics

Karyopharm Therapeutics Inc. (Nasdaq:KPTI) is a clinical-stage pharmaceutical company focused on the discovery and development of novel first-in-class drugs directed against nuclear transport and related targets for the treatment of cancer and other major diseases. Karyopharm's SINE™ compounds function by binding with and inhibiting the nuclear export protein XPO1 (or CRM1). The Company's initial focus is on seeking regulatory approval and commercialization of its lead drug candidate, oral selinexor (KPT-330). To date, over 1,900 patients have been treated with selinexor and it is currently being evaluated in several mid- and later-phase clinical trials across multiple cancer indications, including multiple myeloma in combination with low-dose dexamethasone (STORM) and backbone therapies (STOMP), diffuse large B-cell lymphoma (SADAL), and liposarcoma (SEAL), among others. Karyopharm plans to initiate a pivotal randomized Phase 3 study of selinexor in combination with bortezomib (Velcade®) and low-dose dexamethasone (BOSTON) in patients with multiple myeloma in early 2017. In addition to single-agent and combination activity against a variety of human cancers, SINE™ compounds have also shown biological activity in models of neurodegeneration, inflammation, autoimmune disease, certain viruses and wound-healing. Karyopharm, which was founded by Dr. Sharon Shacham, currently has five investigational programs in clinical or preclinical development. For more information, please visit www.karyopharm.com.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Such forward-looking statements include those regarding the therapeutic potential of and potential clinical development plans for Karyopharm's drug candidates, including the timing of initiation of certain trials and of the reporting of data from such trials, the anticipated impact of the partial clinical hold, timing of FDA review of Karyopharm's response and Karyopharm's plans for obtaining the release of the partial clinical hold. Such statements are subject to numerous important factors, risks and uncertainties that may cause actual events or results to differ materially from the company's current expectations. For example, there can be no guarantee that any of Karyopharm's SINE™ compounds, including selinexor (KPT-330), KPT-8602, Karyopharm's next generation SINE™ compound, or KPT-9274, Karyopharm's first-in-class oral dual inhibitor of PAK4 and NAMPT, or any other drug candidate that Karyopharm is developing will successfully complete necessary preclinical and clinical development phases, that development of any of Karyopharm's drug candidates will continue or that the FDA will release the partial clinical hold in a timely manner or at all. Further, there can be no guarantee that any positive developments in Karyopharm's drug candidate portfolio will result in stock price appreciation. Management's expectations and, therefore, any forward-looking statements in this press release could also be affected by risks and uncertainties relating to a number of other factors, including the following: Karyopharm's results of clinical trials and preclinical studies, including subsequent analysis of existing data and new data received from ongoing and future studies; the content and timing of decisions made by the FDA and other regulatory authorities, investigational review boards at clinical trial sites and publication review bodies; Karyopharm's ability to obtain and maintain requisite regulatory approvals and to enroll patients in its clinical trials; unplanned cash requirements and expenditures; development of drug candidates by Karyopharm's competitors for diseases in which Karyopharm is currently developing its drug candidates; and Karyopharm's ability to obtain, maintain and enforce patent and other intellectual property protection for any drug candidates it is developing. These and other risks are described under the caption "Risk Factors" in Karyopharm's Annual Report on Form 10-K for the year ended December 31 2016, which was filed with the Securities and Exchange Commission

(SEC) on March 16, 2017, 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 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
Darzalex® is a registered trademark of Janssen Biotech, Inc.

Karyopharm Therapeutics Inc.

Consolidated Balance Sheets

(in thousands, except share and per share amounts)

| | <u>December 31,</u> <u>2016</u> | <u>December 31,</u> <u>2015</u> |
|--|------------------------------------|------------------------------------|
| ASSETS | | |
| Current assets: | | |
| Cash and cash equivalents | \$ 49,663 | \$ 58,358 |
| Short-term investments | 79,889 | 117,275 |
| Prepaid expenses and other current assets | 2,084 | 1,967 |
| Total current assets | 131,636 | 177,600 |
| Property and equipment, net | 2,836 | 3,483 |
| Long-term investments | 45,434 | 33,878 |
| Restricted cash | 479 | 482 |
| Total assets | <u>\$ 180,385</u> | <u>\$ 215,443</u> |
| LIABILITIES AND STOCKHOLDERS' EQUITY | | |
| Current liabilities: | | |
| Accounts payable | \$ 4,751 | \$ 3,808 |
| Accrued expenses | 11,362 | 11,023 |
| Deferred rent | 280 | 206 |
| Other current liabilities | 83 | 95 |
| Total current liabilities | 16,476 | 15,132 |
| Deferred rent, net of current portion | 1,666 | 1,946 |
| Total liabilities | <u>18,142</u> | <u>17,078</u> |
| Commitments and contingencies | | |
| Stockholders' equity: | | |
| Preferred stock, \$0.0001 par value; 5,000,000 shares authorized; none issued and outstanding | — | — |
| Common stock, \$0.0001 par value; 100,000,000 shares authorized; 41,887,829 and 35,864,765 shares issued and outstanding at December 31, 2016 and 2015, respectively | 4 | 4 |
| Additional paid-in capital | 528,617 | 455,170 |
| Accumulated other comprehensive loss | (274) | (282) |
| Accumulated deficit | (366,104) | (256,527) |
| Total stockholders' equity | <u>162,243</u> | <u>198,365</u> |
| Total liabilities and stockholders' equity | <u>\$ 180,385</u> | <u>\$ 215,443</u> |

Karyopharm Therapeutics Inc.

CONSOLIDATED STATEMENTS OF OPERATIONS

(in thousands, except share and per share amounts)

| | (Unaudited) | | (Unaudited) | |
|---|--|--------------------|------------------------------------|---------------------|
| | For the Quarter Ended, December 31, | | For the Year Ended December 31, | |
| | 2016 | 2015 | 2016 | 2015 |
| Contract and grant revenue | \$ 47 | \$ 25 | \$ 154 | \$ 250 |
| Operating expenses: | | | | |
| Research and development | 20,671 | 24,064 | 86,938 | 97,744 |
| General and administrative | 6,541 | 5,264 | 23,948 | 21,582 |
| Total operating expenses | <u>27,212</u> | <u>29,328</u> | <u>110,886</u> | <u>119,326</u> |
| Loss from operations | (27,165) | (29,303) | (110,732) | (119,076) |
| Other income (expense): | | | | |
| Interest income | 358 | 250 | 1,284 | 897 |
| Other income (expense) | 11 | 7 | 10 | (2) |
| Total other income, net | <u>369</u> | <u>257</u> | <u>1,294</u> | <u>895</u> |
| Loss before income taxes | <u>(26,796)</u> | <u>(29,046)</u> | <u>(109,438)</u> | <u>(118,181)</u> |
| Provision for income taxes | <u>(139)</u> | <u>—</u> | <u>(139)</u> | <u>—</u> |
| Net loss | <u>\$ (26,935)</u> | <u>\$ (29,046)</u> | <u>\$ (109,577)</u> | <u>\$ (118,181)</u> |
| Net loss per share—basic and diluted | <u>\$ (0.65)</u> | <u>\$ (0.81)</u> | <u>\$ (2.92)</u> | <u>\$ (3.32)</u> |
| Weighted-average number of common shares outstanding used in net loss per share—basic and diluted | <u>41,376,022</u> | <u>35,749,362</u> | <u>37,523,051</u> | <u>35,619,506</u> |

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