



October 12, 2017

## **Karyopharm and Ono Pharmaceutical Co. Ltd. Sign Exclusive License Agreement to Develop and Commercialize Selinexor and KPT-8602 in Japan and Other Countries in Asia**

*– ONO Rights Include Oncology Indications for Selinexor and KPT-8602 in Japan, South Korea, Taiwan, Hong Kong and ASEAN countries —*

*– Karyopharm to Receive ¥2.5 billion (US\$22.3 Million) Upfront; Total Deal Value up to Approximately US\$193.0 million with Karyopharm Eligible to Receive up to ¥19.15 billion (US\$ 170.7 Million) in Future Milestones, Plus Royalties —*

NEWTON, Mass. and OSAKA, Japan, Oct. 12, 2017 (GLOBE NEWSWIRE) -- Karyopharm Therapeutics Inc. (Nasdaq:KPTI) (Karyopharm) and Ono Pharmaceutical Co., Ltd. (ONO), today announced their entry into an exclusive license agreement for the development and commercialization of selinexor, Karyopharm's lead, novel, oral Selective Inhibitor of Nuclear Export (SINE™) compound, and KPT-8602, Karyopharm's second-generation oral SINE™ compound. The agreement includes the development of selinexor and KPT-8602 for the diagnosis, treatment and/or prevention of all human oncology indications in Japan, South Korea, Taiwan, Hong Kong, and ASEAN countries (the Territory).

Under the terms of the agreement, Karyopharm will receive a one-time upfront payment of ¥2.5 billion (approximately US\$22.3 million) from ONO and retains all rights to selinexor and KPT-8602 outside the Territory. Karyopharm is eligible to receive up to an additional ¥19.15 billion (approximately US\$170.7 million at the current exchange rate) if specified future development and commercial milestones are achieved by ONO. Karyopharm is also eligible to receive low double-digit royalties based on future net sales of selinexor and KPT-8602 in the Territory. In exchange, ONO will receive exclusive rights to develop and commercialize both compounds in the Territory, at its own cost and expense. ONO will also have the ability to participate in any global clinical study of selinexor and KPT-8602, and will bear the cost and expense for patients enrolled in clinical studies in the Territory.

"We are very delighted to collaborate on the development of selinexor and KPT-8602, an early development stage XPO-1 inhibitor with Karyopharm, a leading pharmaceutical company focused on the research and development of novel first-in-class drugs in the oncology field," said Gyo Sagara, President, Representative Director of ONO. "We believe both products will present a new treatment option to patients suffering from devastating cancers in Asian countries."

"Given ONO's established leadership in oncology, including Opdivo® (nivolumab) and Kyprolis® (carfilzomib) in Japan, we believe there is no company better suited to advance both selinexor and KPT-8602 in Japan and the other licensed territories," said Michael G. Kauffman, MD, PhD, Chief Executive Officer of Karyopharm. "ONO is well-known and widely respected for its clinical development and commercial expertise, and this partnership provides important validation for both compounds, while allowing us to remain focused on executing our late-phase selinexor trials and pursue regulatory approval in the United States and European Union. We look forward to working with the ONO team to advance both compounds with the goal of rapidly bringing them to patients who are in need of new treatment options."

### **About Selinexor**

Selinexor (KPT-330) is a first-in-class, oral Selective Inhibitor of Nuclear Export / SINE™ compound. Selinexor functions by binding with and inhibiting the nuclear export protein XPO1 (also called CRM1), leading to the accumulation of tumor suppressor proteins in the cell nucleus. This reinitiates and amplifies their tumor suppressor function and is believed to lead to the selective induction of apoptosis in cancer cells, while largely sparing normal cells. To date, over 2,200 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 in multiple myeloma in a pivotal, randomized Phase 3 study in combination with Velcade® (bortezomib) and low-dose dexamethasone (BOSTON), in combination with low-dose dexamethasone (STORM) and backbone therapies (STOMP), and in diffuse large B-cell lymphoma (SADAL), and liposarcoma (SEAL), among others. Additional Phase 1, Phase 2 and Phase 3 studies are ongoing or currently planned, including multiple studies in combination with one or more 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).

### **About KPT-8602**

KPT-8602 is a second generation oral SINE™ compound. KPT-8602 functions by binding to and inhibiting the nuclear export protein XPO1 (also called CRM1), leading to the accumulation of tumor suppressor proteins in the cell nucleus. KPT-8602 has demonstrated minimal brain penetration in animals, which has been associated with reduced toxicities in preclinical studies while maintaining potent anti-tumor effects.

### **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). 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 several investigational programs in clinical or preclinical development. For more information, please visit [www.karyopharm.com](http://www.karyopharm.com).

### **About Ono Pharmaceutical Co., Ltd.**

Ono Pharmaceutical Co., Ltd., headquartered in Osaka, Japan, is an R&D-oriented pharmaceutical company committed to creating innovative medicines in specific areas. It focuses especially on the diabetes and oncology areas. For more information, please visit the company's website at <http://www.ono.co.jp/eng/index.html>.

### **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 potential to receive milestone and royalty payments under the license agreement with ONO; the success of Karyopharm's arrangement with ONO and the parties' ability to work effectively together; and the therapeutic potential of and potential clinical development plans for Karyopharm's drug candidates. Such statements are subject to numerous important factors, risks and uncertainties that may cause actual events or results to differ materially from Karyopharm's current expectations. For example, there can be no guarantee that any of Karyopharm's SINE™ compounds, including selinexor (KPT-330), will successfully complete necessary preclinical and 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 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 ability of Karyopharm or ONO to fully perform their respective obligations under the license agreement; 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 impact volatility in currency exchange rates, 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; Karyopharm's ability to obtain and maintain requisite regulatory approvals and to enroll patients in its clinical trials; unplanned cash requirements and expenditures; development of drug candidates by Karyopharm's competitors for diseases in which Karyopharm is currently developing its drug candidates; and Karyopharm's ability to obtain, maintain and enforce patent and other intellectual property protection for any drug candidates it is developing. These and other risks are described under the caption "Risk Factors" in Karyopharm's Quarterly Report on Form 10-Q for the quarter ended June 30, 2017, which was filed with the Securities and Exchange Commission (SEC) on August 8, 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, 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.

Opdivo® is a registered trademark of Bristol-Meyers Squibb Company.

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

Velcade® is a registered trademark of Takeda Pharmaceutical Company Limited

### **Contacts:**

KARYOPHARM THERAPEUTICS, INC.

Investors:

Kimberly Minarovich

(646) 368-8014

[kimberly@argotpartners.com](mailto:kimberly@argotpartners.com)

Gus Jenkins

(646) 351-1067

[gus@argotpartners.com](mailto:gus@argotpartners.com)

Media:

Eliza Schleifstein

(917) 763-8106

[eliza@argotpartners.com](mailto:eliza@argotpartners.com)

ONO PHARMACEUTICAL CO., LTD.

Corporate Communications

[public\\_relations@ono.co.jp](mailto:public_relations@ono.co.jp)