Karyopharm Receives Orphan Drug Designation from FDA for Eltanexor for the Treatment of Myelodysplastic Syndromes

NEWTON, Mass., Jan. 24, 2022 /PRNewswire/ -- Karyopharm Therapeutics Inc. (Nasdaq: KPTI), a commercial-stage pharmaceutical company pioneering novel cancer therapies, today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation for eltanexor, a novel oral, Selective Inhibitor of Nuclear Export (SINE) compound, for the treatment of myelodysplastic syndromes (MDS). MDS are a group of diseases characterized by ineffective production of the components of the blood due to poor bone marrow function with a risk of progression to acute myeloid leukemia.

Karyopharm is currently investigating eltanexor in an ongoing open-label Phase 1/2 study as a single-agent or in combination with approved and investigational agents in patients with several types of hematologic and solid tumor cancers (KCP-8602-801; NCT02649790). Previously, Karyopharm reported positive data from an investigator-sponsored Phase 1 study evaluating single-agent eltanexor in patients with hypomethylating agent (HMA)-refractory MDS, where eltanexor demonstrated a 53% overall response rate and median overall survival of 9.9 months. This compares favorably to historical survival of four to six months for HMA-refractory MDS patients.

Approximately 15,000 people are diagnosed with intermediate-to-high risk MDS each year in the U.S[1]. HMAs are the current standard of care for newly diagnosed, higher-risk MDS patients. However, only 40-60% of patients respond, with these responses typically lasting less than two years.[2] The prognosis in HMA-refractory disease is poor, with a median overall survival of four to six months.[3,4] There are currently no approved therapies for HMA- refractory MDS.

"We are pleased to receive the FDA's orphan drug designation for eltanexor in MDS and believe it reinforces eltanexor's potential to improve clinical outcomes for patients with HMA-refractory MDS," said Richard Paulson, President and Chief Executive Officer of Karyopharm. "We are focused on advancing our ongoing clinical trials and remain steadfast in our commitment to bringing this new treatment option to patients and their families."

Orphan drug designation by the FDA is granted to promote the development of drugs that target conditions affecting 200,000 or fewer U.S. patients annually and are expected to provide a significant therapeutic advantage over existing treatments. Orphan designation qualifies a company for certain incentives that apply across all stages of drug development, including the potential for seven years of market exclusivity following marketing approval, tax credits on qualified U.S. clinical trials, eligibility for orphan drug grants, and exemption from certain administrative fees.

About Eltanexor

Eltanexor (KPT-8602) is an investigational novel SINE compound that, like selinexor, functions by binding with, and inhibiting, the nuclear export protein, XPO1, 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.

In preclinical models, eltanexor has a broad therapeutic window with minimal penetration of the blood brain barrier and, therefore, has the potential to serve as another SINE compound for cancer indications. Following oral administration, animals treated with eltanexor show lower percentage of body weight loss and improved food consumption than animals similarly treated with selinexor. This allows more frequent dosing of eltanexor, enabling a longer period of exposure than is possible with selinexor.

Eltanexor is an investigational medicine and has not been approved by the United States Food and Drug Administration or any other regulatory agency.

About Karyopharm Therapeutics

Karyopharm Therapeutics Inc. (Nasdaq: KPTI) is a commercial-stage pharmaceutical company pioneering novel cancer therapies. Since its founding, Karyopharm has been the industry leader in oral Selective Inhibitor of Nuclear Export (SINE) compound technology, which was developed to address a fundamental mechanism of oncogenesis: nuclear export dysregulation. Karyopharm's lead SINE compound and first-in-class, oral exportin 1 (XPO1) inhibitor, XPOVIO® (selinexor), is approved in the U.S. and marketed by the Company in three oncology indications and has received regulatory approvals in various indications in a growing number of ex-U.S. territories and countries, including Europe and the United Kingdom (as NEXPOVIO®) and China. Karyopharm has a focused pipeline targeting multiple high unmet need cancer indications, including in endometrial cancer, myelodysplastic syndromes and myelofibrosis. For more information about our people, science and pipeline, please visit www.karyopharm.com, and follow us on Twitter at@Karyopharm and LinkedIn.

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 ability of selinexor or eltanexor to treat patients with multiple myeloma, diffuse large B-cell lymphoma, solid tumors and other diseases and expectations related to future clinical development and potential regulatory submissions of selinexor or eltanexor. 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 grant confirmatory approval in the European Union based on the BOSTON study in adult patients with multiple myeloma; or that any of Karyopharm's drug candidates, including selinexor and eltanexor, 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 obtain and 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 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, 2021, which was filed with the Securities and Exchange Commission (SEC) on November 3, 2021, 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.

References:

[1] Clarivate/DRG Myelodysplastic Syndromes Landscape & Forecast (Nov 2020).

[2] Gil-Perez A. Ther Adv Hematol. 2019 doi:10.1177/2040620719847059.

[3] Jabbour E, Cancer. 2010;116(16):3830-4.

[4] Prébet T. J Clin Oncol. 2011;29:3322-7.

SOURCE Karyopharm Therapeutics Inc.

For further information: Investors: Argot Partners, 212.600.1902, karyopharm@argotpartners.com; Media: Sarah Connors, Vice President, Head of Corporate Communications, Karyopharm, 508.654.2277, sarah.connors@karyopharm.com

---

https://investors.karyopharm.com/2022-01-24-Karyopharm-Receives-Orphan-Drug-Designation-from-FDA-for-Eltanexor-for-the-Treatment-of-Myelodysplastic-Syndromes