

Karyopharm Therapeutics Initiates Treatment of Patients with Acute Myeloid Leukemia (AML) in Phase 1 Trial with Oral KPT-330, the First Selective Inhibitor of Nuclear Export (SINE)

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Karyopharm Therapeutics Inc., a leader in the new field of nuclear transport modulators, announced the dosing of patients with AML in the ongoing Phase 1 clinical trial of KPT-330 in patients with advanced hematologic malignancies. KPT-330 is the first oral SINE Exportin 1 (XPO1/CRM1) antagonist to enter human studies. XPO1 mediates the nuclear export and inactivation of key tumor suppressor proteins. XPO1 blockade causes nuclear accumulation and functional activation of the tumor suppressor proteins, leading to potent and selective tumor cell apoptosis while sparing normal cells. Recent publications from the laboratories of Dr. Romero Garzon at the Ohio State University (Columbus, OH) and Dr. Thomas Look at the Dana Farber Cancer Center (Boston, MA) demonstrate the potent activity of SINE compounds in preclinical models of AML.

Patients with AML are now eligible to enter the KPT-330 Phase 1 trial in hematologic malignancies (NCT01607892), which is designed to determine the optimal dose of KPT-330 for the treatment of various advanced hematologic malignancies. The trial includes patients with non-Hodgkin's lymphoma, multiple myeloma, Waldenstrom's macroglobulinemia, and chronic lymphocytic leukemia whose disease has relapsed after standard therapies. This study now allows the entry of AML patients with either relapsed/refractory disease or who are not able to receive standard chemotherapies. Sharon Shacham, PhD, MBA, Karyopharm's Founder, Chief Scientific Officer, and President of Research and Development commented, "We are very pleased to expand our enrollment in the ongoing Phase 1 study of KPT-330 to patients with AML whose disease has relapsed after, or who cannot receive, standard chemotherapies. KPT-330 and related SINE compounds have shown promising preclinical activity in several AML models, and we are hopeful that these results translate into benefits for patients with this devastating leukemia."

The study is being conducted in the United States, Toronto, Canada, and Copenhagen, Denmark. NPM Pharma (Canada) is overseeing the trial on behalf of Karyopharm.

About Karyopharm Therapeutics Inc.

Karyopharm Therapeutics Inc. is a clinical-stage pharmaceutical company leading the development of small molecule modulators of nuclear transport. The company was founded by Drs. Sharon Shacham and Michael Kauffman in 2009 and has emerged as a leader in the new field of nuclear transport modulators. Karyopharm's selective inhibitors of nuclear export (SINE) function by trapping multiple tumor suppressor proteins in the nucleus, resulting in anti-cancer activity across multiple tumor types. In collaboration with many academic laboratories, SINEs, targeting the major nuclear exporter XPO1 (also called CRM1), exert robust anti-cancer activity in diverse preclinical models of cancer. The lead SINE KPT-330 is in two Phase 1 clinical studies for advanced solid tumor and hematologic malignancies. The related SINE KPT-335 is being evaluated as an oral treatment for dogs with Non-Hodgkin's Lymphoma, one of the most common canine cancers. The Company is also testing SINEs in autoimmune, viral and dermatologic disorders. Karyopharm Therapeutics is located in Natick, Massachusetts.

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