Karyopharm Therapeutics Announces Preclinical Data for Selinexor (KPT-330) in Acute Myeloblastic Leukemia (AML) and Acute Lymphoblastic Leukemia (ALL) Models at FASEB Science Research Conferences

Karyopharm Therapeutics Announces Preclinical Data for Selinexor (KPT-330) in Acute Myeloblastic Leukemia (AML) and Acute Lymphoblastic Leukemia (ALL) Models at FASEB Science Research ConferencesNatick, Mass. – August 7, 2013 – Karyopharm Therapeutics Inc., a clinical-stage pharmaceutical company focused on developing first-in-class nuclear transport modulators for the treatment of cancer and other major diseases, announced that a poster titled “Novel inhibitors of CRM1/XPO1 nuclear exporter exhibit [striking] anti-leukemic activity against AML and T-ALL cells while sparing normal hematopoietic cells” was presented today at the Federation of American Societies for Experimental Biology (FASEB) conference on Hematological Malignancies being held in Saxtons River, Vermont, this week as part of the FASEB 2013 Summer Research Conferences series. The poster provided preclinical data showing evidence that Karyopharm’s proprietary SINE Exportin 1 (XPO1 or CRM1) antagonists induce apoptosis of leukemic cells in models of AML and ALL.

“As we continue to make advances in the clinical development of Selinexor as an anti-tumor agent, this preclinical research further validates XPO1 inhibition as a targeted mechanism to induce apoptosis across many types of cancer cells,” stated Karyopharm Founder, Chief Scientific Officer, and President of Research and Development, Sharon Shacham, Ph.D., M.B.A. “These data being presented by Dr. Etchin and her colleagues at Dana-Farber further build on prior published preclinical data as well as data from the Phase 1 trial in solid tumor patients that was presented at ASCO this year.”

The results being presented demonstrate that SINE compounds induce the selective killing of leukemic cells by apoptosis, with minimal effects on normal white blood cells, in vitro and in animal models. In addition, the data show that the oral SINE compound Selinexor targets both “usual” leukemic cells as well as leukemia-initiating cells (LICs), also called “leukemia stem cells.” The preclinical study is being conducted at Dana-Farber Cancer Institute, Harvard Medical School in Boston, Massachusetts by Julia Etchin, Ph.D. and her colleagues from the laboratory of Dr. A. Thomas Look.

Dr. Look commented, “These results confirm and expand upon data from our previous studies by demonstrating that SINE compounds can kill leukemia initiating cells and usual leukemia cells, all while sparing normal hematopoietic stem cells.”

Selinexor is the first oral SINE XPO1 antagonist to enter human clinical trials. Karyopharm is currently testing Selinexor in two separate Phase 1 clinical trials in patients with relapsed or refractory hematological malignancies and advanced or metastatic solid tumor malignancies, along with a Phase 1b food-effect study in patients with refractory sarcomas.

More information about the ongoing Phase 1 clinical trial of Selinexor in AML and other hematologic malignancies is available at http://clinicaltrials.gov/show/NCT01607892. The information contained in, or accessible through, the clinicaltrials.gov website is not part of this press release and we have included this website solely as an inactive textual reference.

About Karyopharm

Karyopharm Therapeutics Inc. is a clinical-stage pharmaceutical company founded by Drs. Sharon Shacham and Michael Kauffman 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. Preliminary evidence of anti-tumor activity across multiple tumor types has been observed by Karyopharm in preclinical studies and Phase 1 clinical trials. Karyopharm’s lead SINE product candidate Selinexor (KPT-330) is in three Phase 1 clinical trials for advanced solid tumor and hematologic malignancies, including a Phase 1b food-effect study in patients that have metastatic, locally advanced or locally recurrent soft tissue or bone sarcomas. The related SINE Verdinexor (KPT-335) has completed a pivotal study 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 is located in Natick, Massachusetts.
