

# Karyopharm Therapeutics Announces 15 Presentations on Selective Inhibitors of Nuclear Export (SINE) in Solid and Hematologic Malignancy Models at the American Association of Cancer Research (AACR) Meeting

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Karyopharm Therapeutics Inc., a leader in the new field of nuclear transport modulators, announces fifteen (15) poster presentations covering its Selective Inhibitors of Nuclear Export (SINE), oral small molecule XPO1/CRM1 antagonists, at the AACR meeting on April 6 – 13, 2013, in Washington DC. Karyopharm's lead oral XPO1 inhibitor KPT-330 is currently in Phase 1 testing in adult patients with advanced hematologic and solid tumors (NCT01607892, NCT01607905). Karyopharm's related oral XPO1 inhibitor KPT-335 is currently in a potentially pivotal study in dogs with spontaneous non-Hodgkin's lymphomas.

Karyopharm's founder and Chief Scientific Officer Dr. Sharon Shacham commented, "At this AACR meeting, we are fortunate enough to share the results of our ongoing collaborative and internal work on the mechanisms of action and broad anti-tumor activity of SINE compounds for the treatment of a variety of malignant conditions. We believe that these data strengthen the basis for our two Phase 1 first-in-human clinical studies in adults with either solid tumor or hematologic malignancies, and support the future combination of SINEs with other forms of cancer therapy."

The AACR presentations are being made by academic collaborators studying the use of Karyopharm's novel SINEs, along with Karyopharm scientists, and cover the treatment of a variety of adult and pediatric solid tumor and hematologic malignancies as follows:

Drs. Yang Chen and Rachel Altura from the Brown University Medical School will present "Inhibition of the nuclear transport protein CRM1 induces human breast cancer cell death by regulating survivin degradation" (Poster #853; Section 35) on Sunday, April 7 at 1:00-5:00 PM in Halls A-C.

Dr. Yosef Landesman of Karyopharm Therapeutics will present "Deciphering mechanisms of drug sensitivity and resistance to Selective Inhibitors of Nuclear Export (SINE)" (Poster #875; Section 37) on Sunday, April 7 at 1:00-5:00 PM in Halls A-C.

Drs. Joel Turner and Daniel Sullivan from the Moffitt Cancer Center will present "Combination therapy of human multiple myeloma using proteasome and CRM1 inhibitors" (Poster #2066; Section 37) on Monday, April 8 at 1:00-5:00 PM in Halls A-C.

Drs. Tami Rashal and Nir Peled from Sheba Medical Center, Israel, will present "Combination Therapy with KPT-SINE (selective inhibitors of nuclear export) with radiotherapy have additive effects on non-small cell lung cancer (NSCLC) cells in vitro and in vivo" (Poster #2075; Section 37) on Monday, April 8 at 1:00-5:00 PM in Halls A-C.

Drs. Yu-Tzu Tai and Ken Anderson from the Dana Farber Cancer Center will present "The nuclear export protein CRM1 (XPO1) regulates multiple myeloma cell growth, osteoclastogenesis and myeloma-induced osteolysis" (Poster #2142; Section 40) on Monday, April 8 at 1:00-5:00 PM in Halls A-C.

Drs. Chen and Martignetti from the Mount Sinai School of Medicine will present "Increased overall survival in platinum-resistant ovarian cancer: paradigmatic use of novel SINE (selective inhibitor of nuclear export), which restores p53 nuclear localization and activation" (Poster #2163; Section 41) on Monday, April 8 at 1:00-5:00 PM in Halls A-C.

Drs. Attiyeh and Maris from Children's Hospital of Philadelphia, will present "Targeted inhibition of Chromosomal Maintenance Region Protein (CRM1) potently suppresses growth of human neuroblastoma cell line models" (Poster #2756; Section 18) on Tuesday, April 9, at 8:00 AM – 12:00 PM in Halls A-C.

Drs. Giovanni Gravina and Claudio Festuccia of University of L'aquila, Italy, will present "CRM1 Selective Inhibitors of Nuclear Export (SINE) reduce the incidence of tumor spreading and improve overall survival in preclinical models of prostate cancer" (Poster #3440; Section 44) on Tuesday, April 9, at 8:00 AM – 12:00 PM in Halls A-C.

Drs. Jennifer Yang and Greg Lesinski of the Ohio State University will present "Novel small molecule CRM1 inhibitors induce nuclear accumulation of p53, phosphorylated ERK and apoptosis in human melanoma cells" (Poster #3443; Section 44) on Tuesday, April 9, at 8:00 AM – 12:00 PM in Halls A-C.

Drs. Xiaohong Han of the Peking Union Medical College (PUMC), Beijing, will present "Novel(SINE CRM1 antagonists for non-

small cell lung cancer (NSCLC) in vitro and in vivo” (Poster #3444; Section 44) on Tuesday, April 9, at 8:00 AM – 12:00 PM in Halls A-C.

Drs. Asfar Azmi and Ramzi Mohammed of Wayne State University will present “Selective Inhibitors of Nuclear Export (SINE) for cancer therapy: from bench to bedside” (Poster #3445; Section 44) on Tuesday, April 9, at 8:00 AM – 12:00 PM in Halls A-C.

Dr. Dilara McCauley of Karyopharm Therapeutics will present “Selective Inhibitors of Nuclear Export (SINE) display single agent efficacy against gastric (NCI-N87) and colon (HCT-116) xenografts” (Poster #4336; Section 36) on Tuesday, April 9, at 1:00 – 5:00 PM in Halls A-C.

Drs. Peter Houghton and Malcolm Smith will present “Pediatric Preclinical Testing Program (PPTP) stage 1 evaluation of the XPO1/CRM1 inhibitor KPT-330” (Late Breaking Poster #LB- 354; Section 48) on Wednesday, April 10, at 8:00 AM – 12:00 PM, in Halls A-C.

Drs. Jayasree Nair and Gary Schwartz of the Memorial Sloan Kettering Cancer Center will present “KPT-330, a selective small molecule inhibitor of nuclear export, is active in bone and soft tissue sarcoma” (Poster #5210; Section 25) on Wednesday, April 10, at 8:00 AM – 12:00 PM, in Halls A-C.

Drs. Takahito Miyake and Anil Sood of the MD Anderson Cancer Center will present “Therapeutic targeting of CRM1 in ovarian cancer” (Poster #5541; Section 38) on Wednesday, April 10, at 8:00 AM – 12:00 PM, in Halls A-C.

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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