Oncternal Therapeutics Initiates Patient Dosing in Phase 1 Clinical Trial of TK216 in Ewing Sarcoma

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SAN DIEGO, August 30, 2016 — Oncternal Therapeutics, Inc., a clinical-stage biotechnology company developing first-in-class therapies for rare and common malignancies, today announced that the first patient has been dosed in a Phase 1 clinical trial of TK216 in Ewing sarcoma. TK216 is a small molecule therapeutic candidate that inhibits the biological activity of *ets*-family transcription factor oncoproteins, which are the main disease drivers in Ewing tumors. The trial will evaluate safety and efficacy of TK216 in patients with relapsed or refractory Ewing sarcoma.

TK216 recently received Orphan Drug Designation from the U.S. Food and Drug Administration (FDA) for treating Ewing sarcoma, a rare pediatric cancer, and FDA Fast Track Designation for treating Ewing patients who are relapsed or refractory to standard therapies.

“Ewing sarcoma is a very serious bone cancer affecting children and young adults and the effects can be devastating,” said James Breitmeyer, M.D., Ph.D., Oncternal’s President and CEO. “These patients have an urgent need for additional treatment options and TK216 represents the first potential new therapy for Ewing sarcoma in many decades. We are delighted that enrollment and dosing are now underway in this important clinical trial.”

“The seminal research into targeting the Ewing oncogene by inhibiting the protein interactions of EWS-FLI1 was conducted at Georgetown,” said Jeffrey Toretsky, M.D., Professor and Molecular Oncology Program Co-Leader at the Lombardi Comprehensive Cancer Center, Georgetown University. “My colleagues and I are gratified to see this novel approach translated into a therapeutic candidate, which is now being evaluated in a clinical trial for Ewing sarcoma patients.”

More information on the Phase 1 trial can be found at ClinicalTrials.gov, including participating sites: Memorial Sloan Kettering Cancer Center in New York City, UCLA Jonsson Comprehensive Cancer Center in Los Angeles, and MD Anderson Cancer Center in Houston.

About TK216

TK216 is a first-in-class small molecule that inhibits the biological activity of *ets*-family transcription factor oncoproteins in a variety of tumor types, stopping cancer cell growth and tumor formation. In Ewing sarcoma, it is designed to target a single and well-characterized genetic mutation that causes the disease. TK216 is being developed collaboratively by Georgetown University and Oncternal. The original work took place at Georgetown Lombardi Comprehensive Cancer Center in the lab of Jeffrey A. Toretsky, M.D., who was one of the inventors of the underlying technology and related intellectual property owned by Georgetown. It is licensed to Oncternal for further development as a potential therapeutic agent for cancer, including treatment of Ewing sarcoma. Oncternal and Georgetown are also planning clinical studies of TK216 in glioblastoma, prostate cancer and leukemia in the next year.