TK216 Receives Orphan Drug Designation for the Treatment of Ewing Sarcoma, a Rare Pediatric Cancer

July 13, 2016

SAN DIEGO, July 13, 2016 — Oncternal Therapeutics, Inc., a clinical-stage oncology company developing first-in-class therapies for rare and common malignancies, today announced that TK216 has received Orphan Drug Designation from the U.S. Food and Drug Administration (FDA)for the treatment of Ewing sarcoma. Ewing sarcoma is a rare pediatric cancer comprised of highly malignant, undifferentiated tumors of bone. It can develop in almost any bone in the body, however soft tissue tumors are not uncommon. TK216 is a first-in-class small molecule inhibiting *ets*-family transcription factor oncoproteins, which are the main disease drivers in Ewing tumors. Oncternal is in the process of initiating a first-in-human Phase 1 trial of TK216 in relapsed or refractory Ewing sarcoma. The company recently received Fast Track Designation from the FDA for TK216 in this indication.

The FDA's Orphan Drug Designation program provides orphan status to drugs and biologics which meet certain criteria and treat, diagnosis or prevent rare diseases/disorders affecting fewer than 200,000 people in the U.S. Orphan designation qualifies the developing company for incentives such as tax credits and fee waivers.

"Ewing sarcoma is a rare pediatric cancer, and most patients who present with metastatic disease or fail first-line therapy face a poor prognosis. These young patients are in urgent need of better treatment options," said James Breitmeyer, M.D., Ph.D., Oncternal's President and CEO. "We are pleased to have support from the FDA through the Orphan Drug Designation program as we initiate our first clinical trial of TK216, treating relapsed or refractory Ewing sarcoma patients 12 years and older."

90% of Ewing sarcoma cases occur in patients between the ages of 5 and 25 years, and two thirds are found in those between 10 and 20 years of age. The median patient age is 15 years. Ewing sarcoma is a rare disease, with approximately 600-800 new cases diagnosed annually in the U.S., an incidence rate that has remained fairly constant over the last 30 years at nearly three cases per 1 million people. The 5-year survival rate for patients with localized disease is approximately 75%, but if the tumor has spread or metastasized at the time of diagnosis, the 5-year survival rate drops to 30%.

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 the inventor 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.