Oncternal Therapeutics Announces Presentation of Case Study of TK216 in Ewing Sarcoma Given at the Children's Oncology Group Meeting

September 17, 2019

Sustained objective response reported for patient with challenging pediatric cancer

SAN DIEGO--(BUSINESS WIRE)--Sep. 17, 2019-- Oncternal Therapeutics, Inc. (Nasdaq: ONCT), a clinical-stage biopharmaceutical company focused on the development of novel oncology therapies, today announced that Paul A. Meyers, M.D., Chief, Pediatric Sarcoma Service and Vice Chair for Clinical Affairs of Memorial Sloan Kettering Cancer Center, presented a case study of a patient with Ewing sarcoma who achieved a sustained response following treatment with Oncternal's investigational product candidate, TK216, in an ongoing Phase 1, first-in-human clinical trial. The presentation entitled, "TK216 for the Treatment of Ewing Sarcoma," was given at the Fall Children's Oncology Group (COG) Meeting.

Dr. Meyers reported that the patient, who had a history of Ewing sarcoma with pulmonary metastases, had recurrent disease despite multiple courses of chemotherapy, radiation, bevacizumab, pazopanib and surgery. Following two cycles of TK216 therapy given as a single agent, the patient achieved a confirmed objective response, which included resolution of several pulmonary lesions. This response has been sustained and has continued at six months of treatment, with the patient receiving TK216 plus vincristine in subsequent treatment courses. The final remaining residual tumor nodule which was less than one centimeter in diameter was later surgically removed, leading to a surgical complete remission. Treatment with TK216 has been well-tolerated by this patient.

"I am encouraged that this patient has had a sustained, impressive response on the TK216 study," said Dr. Meyers. "There is a high unmet medical need for new options to treat Ewing sarcoma, which is a rare cancer that affects mostly pediatric patients and young adults and has been very challenging to treat effectively."

"We are pleased by the results reported by Dr. Meyers and look forward to examining what we believe could be a recommended dosing regimen of TK216 in a larger number of patients with Ewing sarcoma," said James Breitmeyer, M.D., Ph.D., Oncternal's President and CEO.

About TK216

TK216 is an investigational, potentially first-in-class small molecule that is designed to inhibit the biological activity of E26 transformation-specific (ETS) transcription factor oncoproteins including fusion proteins. Tumorigenic gene fusions involving ETS factors are frequently found in tumors such as Ewing sarcoma and prostate cancer, and ETS factors are often overexpressed in other tumors such as prostate cancer, and acute myeloid leukemia (AML). TK216 was developed based on discoveries of Jeffrey Toretsky, M.D. and his team at Georgetown University, who found inhibitors of EWS-FLI1 using a novel chemical screening assay. In preclinical models, TK216 binds to EWS-FLI1 and blocks the interaction between ETS family members and RNA helicase A leading to tumor cell apoptosis.

About the Study

TK216 is being evaluated in a Phase 1 clinical study as a single agent and in combination with vincristine in patients with relapsed or refractory Ewing sarcoma, a rare pediatric cancer that has historically been very challenging to treat effectively, particularly for recurrent and metastatic disease. A dose-finding arm of this study is nearing completion, after which Oncternal intends to begin enrolling patients in an expansion cohort of the study to evaluate the clinical response of treatment with TK216 in combination with vincristine, an approved chemotherapy agent. This multi-center study is actively enrolling patients at six clinical trial centers across the U.S. Additional information about the TK216 study may be accessed at ClinicalTrials.gov (NCT02657005).

About Oncternal Therapeutics

Oncternal Therapeutics is a clinical-stage biopharmaceutical company focused on developing product candidates for the treatment of cancers with critical unmet medical need. Oncternal focuses drug development on promising yet untapped biological pathways implicated in cancer generation or progression. The pipeline includes cirmtuzumab, a monoclonal antibody designed to inhibit the ROR1 receptor, a type I tyrosine kinase-like orphan receptor that is expressed by many cancers but not by normal tissues and is associated with cancer stemness, that is being evaluated in a Phase 1/2 clinical trial in combination with ibrutinib for the treatment of chronic lymphocytic leukemia (CLL) and mantle cell lymphoma (MCL), and TK216, a small-molecule compound that is designed to inhibit E26 transformation specific (ETS) family oncoproteins, which is being evaluated in a Phase 1 clinical trial for patients with Ewing sarcoma alone and in combination with vincristine chemotherapy. In addition, Oncternal has a CAR-T product candidate that targets ROR1, which is currently in preclinical development as a potential treatment for hematologic cancers and solid tumors. More information is available at www.oncternal.com.

Forward-Looking Information

Oncternal cautions you that statements included in this press release that are not a description of historical facts are forward-looking statements. In some cases, you can identify forward-looking statements by terms such as "may," "will," "should," "expect," "plan," "anticipate," "could," "intend," "target," "project," "contemplates," "believes," "estimates," "predicts," "potential" or "continue" or the negatives of these terms or other similar expressions. These statements are based on the Company's current beliefs and expectations. Forward looking statements include statements regarding Oncternal's beliefs, goals, intentions and expectations, and include Oncternal's expectation that it is nearing completion of the dose-finding portion of its Phase 1 clinical trial and intends to begin enrolling patients in an expansion cohort thereafter; Oncternal's belief that TK216 may address a high unmet medical need, by inhibiting the interaction of between ETS family members and RNA helicase A, thereby shutting down excessive cell proliferation. Forward looking statements are subject to risks and uncertainties, which include, but are not limited to: uncertainties associated with the

clinical development and process for obtaining regulatory approval of TK216 and Oncternal's other product candidates, including potential delays in the commencement, enrollment and completion of clinical trials; the risk that the results seen in a case study of one patient may not predict the results seen in other patients in the clinical trial, including the possibility that there may not be additional sustained responses from other patients in the study; the risk that interim results of a clinical trial do not necessarily predict final results and that one or more of the clinical outcomes may materially change as patient enrollment continues, following more comprehensive reviews of the data, as follow-up on the outcome of any particular patient continues, and as more patient data become available; the risk that unforeseen adverse reactions or side effects may occur in the course of developing and testing product candidates such as TK216 and Oncternal's other product candidates; the risk that Oncternal may be unable to obtain sufficient additional capital to continue to advance the development of TK216 and its other product candidates; and other risks described in the Company's prior press releases as well as in public periodic filings with the U.S. Securities & Exchange Commission. All forward-looking statements in this press release are current only as of the date hereof and, except as required by applicable law, Oncternal undertakes no obligation to revise or update any forward-looking statement, or to make any other forward-looking statements, whether as a result of new information, future events or otherwise. All forward-looking statements are qualified in their entirety by this cautionary statement. This caution is made under the safe harbor provisions of the Private Securities Litigation Reform Act of 1995.

View source version on businesswire.com: https://www.businesswire.com/news/home/20190917006086/en/

Source: Oncternal Therapeutics

Oncternal Contacts:
Investors
Richard Vincent
858-434-1113
rvincent@oncternal.com

Media
Jason Spark
619-849-6005
jason@canalecomm.com