Oncternal Therapeutics Presented Updated Interim Phase 1/2 Clinical Trial Data for ONCT-216 in Patients with Relapsed/Refractory Ewing Sarcoma at CTOS 2021 Virtual Annual Meeting

November 15, 2021

SAN DIEGO, Nov. 15, 2021 (GLOBE NEWSWIRE) -- Oncternal Therapeutics, Inc. (Nasdaq: ONCT), a clinical-stage biopharmaceutical company focused on the development of novel oncology therapies, today announced updated interim clinical data from the Phase 2 expansion cohort of its ongoing Phase 1/2 clinical trial evaluating ONCT-216 (formerly TK216), an investigational, potentially first-in-class, targeted small-molecule inhibitor of the E26 transformation-specific (ETS) family of oncoproteins, in patients with relapsed or refractory Ewing sarcoma. The data update was delivered in an oral presentation at the Connective Tissue Oncology Society (CTOS) 2021 Virtual Annual Meeting.

Session Title:	Session 12: Ultra-Rare and Translocation Sarcomas
Session Date:	Saturday, November 13, 2021
Presentation Title:	Paper 74 - TK216 FOR EWING SARCOMA- INTERIM PHASE 1/2 RESULTS

A copy of the presentation will be accessible on the Events & Presentations page of the Investors section on the Company's website at investor.oncternal.com.

"We remain encouraged by the two complete responses to ONCT-216 in heavily pre-treated patients with relapsed or refractory Ewing sarcoma, including one patient who had a durable CR for 24 months on treatment, and remains with no evidence of disease off of all treatments for several months," said James Breitmeyer, M.D., Ph.D., Oncternal's President and CEO. "We believe that an intensified dosing schedule, which we are investigating in a new study cohort that is now enrolling, holds promise to address the significant unmet needs for patients suffering from this devastating disease."

ONCT-216 remains generally well tolerated. As of the October 1, 2021 data cutoff date, the most common drug-related adverse events included myelosuppression, fatigue, alopecia, nausea, pyrexia, and decreased appetite. The myelosuppression was primarily neutropenia, which was transient and readily managed. No unexpected off-target toxicities have been observed.

About Ewing sarcoma

Ewing sarcoma is the second most common bone tumor among children and adolescents. The median age at diagnosis of patients with Ewing sarcoma is 15, the incidence is about 3 cases per 1 million per year in children under the age of 20 and about 1.3 cases per 1 million overall in the U.S. and prevalence is about 12 per million people overall in the US. Nearly all Ewing sarcoma cases are driven by translocations of ETS family oncogenes, including 85-90% of cases driven by the EWS-FLI1 fusion, and approximately 10% by EWS-ERG. Patients diagnosed with metastatic disease have five-year survival rates between 18% and 30%. The prognosis for patients with recurrent Ewing sarcoma is particularly poor, and five-year survival after recurrence is approximately 10 to 15%.

About ONCT-216

ONCT-216 is an investigational, potentially first-in-class, targeted small-molecule inhibitor of the E26 transformation-specific (ETS) family of oncoproteins including fusion proteins. Tumorigenic fusion proteins involving the EWS protein and an ETS protein can be found in most cases of Ewing sarcoma. ETS-related translocations or overexpression are also found in many other tumors such as diffuse large B-cell lymphoma (DLBCL), prostate cancer and acute myeloid leukemia (AML). ONCT-216 was developed based on discoveries in the laboratory of Jeffrey Toretsky, M.D., at Georgetown Lombardi Comprehensive Cancer Center, who discovered inhibitors of EWS-FLI1 using a novel chemical screening assay. In preclinical models, ONCT-216 was observed to bind to EWS-FLI1, blocking the interaction between this fusion protein and other transcriptome proteins such as RNA helicase A, leading to tumor cell apoptosis and inhibiting tumor growth in animal models. The U.S. Food and Drug Administration (FDA) has granted Rare Pediatric Disease Designation, Orphan Drug Designation and Fast Track Status to ONCT-216 for the treatment of Ewing sarcoma. ONCT-216 is an investigational medication that has not been approved by the FDA for any indication.

About the Study

ONCT-216 is being evaluated in a Phase 1/2 clinical study as a single agent and in combination with vincristine in heavily pretreated patients with relapsed or refractory Ewing sarcoma, a rare pediatric cancer with no standard treatment available after first-line chemotherapy. The current Phase 2 expansion cohort targeting up to 21 evaluable Ewing sarcoma patients is active and enrolling patients, designed to evaluate clinical responses to single agent ONCT-216 using an optimized dosing regimen, treating for 28 days per cycle, to intensify the amount of ONCT-216 administered over time. This multi-center study is currently enrolling patients at nine clinical trial centers across the U.S. Additional information about the ONCT-216 study may be accessed at ClinicalTrials.gov (NCT02657005).

About Oncternal Therapeutics

Oncternal Therapeutics is a clinical-stage biopharmaceutical company focused on the development of novel oncology therapies 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 clinical pipeline includes zilovertamab (formerly cirmtuzumab) an investigational monoclonal antibody designed to inhibit the ROR1 pathway, a type I tyrosine kinase-like orphan receptor, that is being evaluated in a Phase 1b/2 clinical trial in combination with ibrutinib for the treatment of patients with mantle cell lymphoma (MCL) and chronic lymphocytic leukemia (CLL) and in an investigator-sponsored, Phase 1b clinical trial in combination with paclitaxel for the treatment of women with HER2-negative metastatic or locally advanced, unresectable

breast cancer, as well as a Phase 2 clinical trial of zilovertamab in combination with venetoclax, a Bcl-2 inhibitor, in patients with relapsed/refractory CLL. Oncternal is also developing ONCT-808, a chimeric antigen receptor T cell (CAR-T) therapy that targets ROR1, which is currently in preclinical development as a potential treatment for hematologic cancers and solid tumors. The clinical pipeline also includes ONCT-216 (formerly TK216), an investigational targeted small-molecule inhibitor of the ETS family of oncoproteins, that is being evaluated in a Phase 1/2 clinical trial for patients with Ewing sarcoma alone and in combination with vincristine chemotherapy. The early-stage pipeline also includes ONCT-534 (formerly GTX-534), a dual-action androgen receptor inhibitor, that is in pre-clinical development as a potential treatment for castration resistant prostate cancer and other androgen-receptor dependent diseases. More information is available at https://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 Oncternal’s current beliefs and expectations. Forward-looking statements include statements regarding the potential for ONCT-216 dosing changes to address unmet medical needs. Forward-looking statements are subject to risks and uncertainties inherent in Oncternal’s business, including risks associated with the clinical development and process for obtaining regulatory approval of Oncternal’s product candidates, such as potential delays in the commencement, enrollment and completion of clinical trials; the risk that results seen in a case study of one patient likely will not predict the results seen in other patients in the clinical trial; the risk that interim results of a clinical trial do not 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; and other risks described in Oncternal’s filings with the U.S. Securities and 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.

Investors
Richard Vincent
Chief Financial Officer
858-434-1113
rvincent@oncternal.com

Media
Corey Davis
LifeSci Advisors
212-915-2577
cdavis@lifesciadvisors.com

Source: Oncternal Therapeutics