# Oncternal Therapeutics Receives Rare Pediatric Disease Designation from U.S. Food and Drug Administration for TK216 for Treatment of Ewing Sarcoma

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SAN DIEGO--(BUSINESS WIRE)--Oct. 6, 2020-- Oncternal Therapeutics, Inc. (Nasdaq: ONCT), a clinical-stage biopharmaceutical company focused on the development of novel oncology therapies, today announced that the U.S. Food and Drug Administration (FDA) has granted rare pediatric disease designation for TK216, an investigational potentially first-in-class targeted small-molecule inhibitor of the E26 transformation-specific (ETS) family of oncoproteins, for treatment of Ewing sarcoma.

Under the FDA's rare pediatric disease designation and voucher program, the FDA may grant a priority review voucher to a sponsor who receives a product approval for a "rare pediatric disease," which is defined as a serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years and which either affects fewer than 200,000 people in the U.S., or affects more than 200,000 people in the U.S. but with no reasonable expectation that the cost of developing and making the drug available in the U.S. will be recovered from U.S. sales. Subject to FDA approval of TK216 for the treatment of Ewing sarcoma, Oncternal may be eligible to receive a priority review voucher if the marketing application submitted for the product satisfies certain additional conditions, including approval no later than September 30, 2022 (unless this statutory sunset provision is modified by Congress). If issued, this voucher may be redeemed to receive priority review for a subsequent marketing application or may be sold or transferred to another sponsor.

"The FDA's rare pediatric disease designation of TK216 for treatment of Ewing sarcoma, for which Oncternal had previously received FDA's Orphan Drug and Fast Track designations, underscores the agency's recognition that Ewing sarcoma is a devastating cancer, with a high unmet medical need," said James Breitmeyer, M.D., Ph.D., President and CEO, Oncternal. "An expansion cohort in the clinical trial of TK216 for patients with relapsed/refractory Ewing sarcoma is currently enrolling, and we expect to present additional interim clinical data from our ongoing Phase 1 clinical trial at a scientific conference in the fourth quarter of 2020."

**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, and 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. 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 TK216**

TK216 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 prostate cancer and acute myeloid leukemia (AML). TK216 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, TK216 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 Orphan designation, Fast Track designation, and Rare Pediatric Disease designation to TK216 for the treatment of Ewing sarcoma. TK216 is an investigational medication that has not been approved by the FDA for any indication.

**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 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 1/2 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. The clinical pipeline also includes TK216, an investigational targeted small-molecule inhibitor of the ETS family of oncoproteins, that 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 program utilizing the cirmtuzumab antibody backbone to develop a CAR-T therapy 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 the potential that the FDA may grant a priority review voucher; the anticipated dates for announcing additional clinical data from the ongoing Phase 1 clinical trial in patients with relapsed/refractory Ewing sarcoma; estimated patient population sizes; and other statements regarding

Oncternal's development plans. Forward looking statements are subject to risks and uncertainties inherent in Oncternal's business, which include, but are not limited to: FDA may not grant a priority review voucher, even if TK216 is approved by the FDA; the FDA will not issue a priority review voucher if TK216 is approved after September 30, 2022 (unless this statutory sunset provision is modified by Congress); Oncternal has encountered delays, and may encounter additional delays or difficulties, in enrolling patients in its clinical trials as a result of the COVID-19 pandemic; the COVID-19 pandemic may disrupt Oncternal's business operations, increasing its costs; 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; Oncternal's dependence on the success of cirmtuzumab, TK216 and its other product development programs; the risk that the regulatory landscape that applies to the development program for cirmtuzumab, TK216 and the company's other product candidates may change over time; the risk that the approval of one of Oncternal's product candidates may be blocked for seven years if a competitor obtains approval of the same drug or biologic, as defined by the FDA, or if its product candidate is determined to be contained within the competitor's product for the same indication or disease; the risk that competitors may develop technologies or product candidates more rapidly than Oncternal, or that are more effective than Oncternal's product candidates, which could significantly jeopardize Oncternal's ability to develop and successfully commercialize its product candidates; Oncternal's limited operating history and the fact that it has incurred significant losses, and expects to continue to incur significant losses for the foreseeable future; the risk that the company will have insufficient funds to finance its planned operations and may not be able to obtain sufficient additional financing when needed or at all as required to achieve its goals, which could force the company to delay, limit, reduce or terminate its product development programs or other operations; the risk that the benefits associated with orphan drug designation may not be realized, including that orphan drug exclusivity may not effectively protect a product from competition and that such exclusivity may not be maintained; the risk that, if an orphan designated product, including cirmtuzumab, receives marketing approval for an indication broader than what is designated, it may not be entitled to orphan exclusivity; the possibility that competitors may receive approval of different products for the indication for which an orphan product has exclusivity or obtain approval for the same product but for a different indication for which the orphan product has exclusivity; 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.

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