Oncternal Therapeutics Announces Presentation of ROR1 CAR-T Preclinical Data at 2020 ASCO-SITC Clinical Immuno-Oncology Symposium

February 6, 2020

- Anti-ROR1 CAR-T cell therapy demonstrated expansion, persistence and anti-tumor activity in animal model of human leukemia

SAN DIEGO--(BUSINESS WIRE)--Feb. 6, 2020-- Oncternal Therapeutics, Inc. (Nasdaq: ONCT), a clinical-stage biopharmaceutical company focused on the development of novel oncology therapies, today announced the presentation of preclinical data from its ROR1 chimeric antigen receptor T cell (CAR-T) program at the American Society of Clinical Oncology (ASCO) – Society for Immunotherapy of Cancer (SITC) meeting in Orlando, Florida. A copy of the poster presentation is available online at www.oncternal.com.

In preclinical studies, anti-ROR1 CAR-T constructs were evaluated in an animal model of human leukemia. A single dose of anti-ROR1 CAR T-cells expanded in treated animals and the chimeric T cells trafficked to the disease sites. By week four, leukemia cells were cleared from major tissue reservoirs, including bone marrow, kidneys and spleen. CAR-T cell-treated animals survived longer than 90 days compared to 21 days for animals in control groups. The CAR-T cells were highly active and detected in mouse tissues more than two months after injection.

This research effort was led by Professor Thomas J. Kipps, M.D., Ph.D., and Charles Prussack, Pharm.D., Ph.D., at the University of California San Diego (UC San Diego) under a research grant from the California Institute of Regenerative Medicine (CIRM).

“It is exciting to see the potent preclinical activity of the ROR1 CAR-T cell therapy and its selectivity in targeting tumors,” said Thomas Kipps, M.D., Ph.D., Professor of Medicine, Evelyn and Edwin Tasch Chair in Cancer Research, and Deputy Director of Research Operations at the UC San Diego Moores Cancer Center. “This CAR-T cell product utilizes the ROR1 binding domain derived from cirmtuzumab (UC-961), a clinical stage antibody that is currently being evaluated in patients with hematological malignancies and solid tumors. The challenges of CAR-T therapies include patient relapses due to the loss of target antigen and safety issues due to targeting of normal cells expressing the antigen. Harnessing cirmtuzumab’s specificity for ROR1 expressed on cancer cells has the potential to improve CAR-T efficacy and safety, and address the high unmet medical need for treating patients with aggressive cancers.”

“We are encouraged by the preclinical results of this ROR1 CAR-T program and look forward to advancing it to clinical testing, initially for treating patients with hematological cancers, potentially in the fourth quarter of this year,” said James Breitmeyer, M.D., Ph.D., Oncternal’s President and CEO.

About ROR1 CAR-T

Oncternal Therapeutics is developing a ROR1-targeting (Receptor tyrosine kinase-like Orphan Receptor 1) chimeric antigen receptor T cell therapy (CAR-T) as a potential treatment for patients with aggressive hematological malignancies or solid tumors, in collaboration with the UC San Diego School of Medicine and the California Institute for Regenerative Medicine (CIRM). The Company’s CAR-T program is based on the binding domain of cirmtuzumab, which is an investigational, potentially first-in-class monoclonal antibody targeting ROR1. Cirmtuzumab is currently being evaluated in a Phase 1/2 clinical trial in combination with ibrutinib for the treatment of patients with chronic lymphocytic leukemia, or CLL, or mantle cell lymphoma, or MCL, and in an investigator-initiated Phase 1 clinical trial in combination with paclitaxel for the treatment of women with metastatic or unresectable breast cancer.

ROR1 is a potentially attractive target for cancer therapy because it is an onco-embryonic antigen – not usually expressed on adult cells, and its expression confers a survival and fitness advantage when reactivated and expressed by tumor cells. Researchers at the UC San Diego School of Medicine discovered that targeting a critical epitope on ROR1 was key to specifically targeting ROR1 expressing tumors. This led to the development of cirmtuzumab, that binds this critical epitope of ROR1, which is highly expressed on many different cancers but not on normal tissues. Because the epitope of ROR1 recognized by cirmtuzumab appears to be restricted to tumor cells, a cirmtuzumab-based CAR-T may be selective in distinguishing cancer from normal tissues. Cirmtuzumab and ROR1 CAR-T cell therapy have not been approved by the U.S. Food and Drug Administration 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 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 chronic lymphocytic leukemia (CLL) and mantle cell lymphoma (MCL), and TK216, an investigational targeted small-molecule inhibitor of the ETS family of oncoproteins, that is being evaluated in a Phase 1b clinical trial for patients with Ewing sarcoma alone and in combination with vincristine chemotherapy. In addition, Oncternal has a program 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 Oncternal’s beliefs, goals, intentions and expectations, and include: Oncternal’s plans for advancing its ROR1 CAR-T cell therapy into

clinical trials for patients with hematological or other cancers, and the anticipated timing for initiating such clinical trials, as well as the company's belief that its ROR1 CAR-T cell therapy could potentially improve CAR-T efficacy and safety and meet unmet medical needs for patients with aggressive cancers. Forward looking statements are subject to risks and uncertainties inherent in Oncternal's business, which include, but are not limited to: the risk that early preclinical testing results of a product candidate or therapy do not necessarily predict the results of later preclinical or clinical testing, and that product candidates or therapies can unexpectedly fail at any stage of preclinical or clinical development; the risk that, because the outcomes of preclinical and clinical studies are highly uncertain, the company cannot reliably predict actual amounts necessary to successfully complete the development and commercialization of its ROR1 CAR-T cell therapy; the risk that unforeseen adverse reactions or side effects may occur in the course of developing and testing product candidates and therapies such as the company's ROR1 CAR-T cell therapy, which could adversely impact the company's ability to complete clinical trials and obtain regulatory approval for such product candidates and therapies; uncertainties associated with the process for obtaining regulatory approval of the ROR1 CAR-T cell therapy and Oncternal's other product candidates; the risk that the regulatory landscape that applies to the development program for the company's ROR1 CAR-T cell therapy and the company's other product candidates may change, which could result in delays or termination of development of such product candidates and therapies or unexpected costs in obtaining regulatory approvals; challenges in developing processes to produce the ROR1 CAR-T cell therapy needed to perform clinical trials, and the costs of production, quality control, testing and maintaining necessary regulatory authorizations associated with such production; 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 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, 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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