



## Oncternal Therapeutics Announces Orphan Drug Designations of Cirmtuzumab ROR1 Antibody for Treatment of Mantle Cell Lymphoma and for Treatment of Chronic Lymphocytic Leukemia

June 30, 2020

SAN DIEGO--(BUSINESS WIRE)--Jun. 30, 2020-- Oncternal Therapeutics, Inc. (Nasdaq: ONCT), a clinical-stage biopharmaceutical company focused on the development of novel oncology therapies, today announced that the United States Food and Drug Administration (FDA) has granted the company orphan drug designations of cirmtuzumab for treatment of mantle cell lymphoma (MCL) and for treatment of chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL). Cirmtuzumab is an investigational anti-ROR1 monoclonal antibody being evaluated in clinical trials in patients with MCL, CLL and HER2-negative breast cancer.

Under the Orphan Drug Act, the FDA may grant orphan drug designation to drugs or biologics intended to treat rare diseases or conditions, which are defined as diseases or conditions that affect fewer than 200,000 people in the United States or that affect more than 200,000 people but where there is no reasonable expectation that the costs of developing and marketing the drug will be recovered through future sales of the drug in the United States. Orphan drug designation for cirmtuzumab qualifies Oncternal for certain benefits including tax credits for qualified clinical trials, exemption from certain FDA application fees, and the potential for market exclusivity upon regulatory approval, if received, for an orphan-designated indication.

"We are pleased to receive orphan drug designations for cirmtuzumab, our potentially first-in-class investigational ROR1 antibody," said James Breitmeyer, M.D., Ph.D., Oncternal's President and CEO. "We are excited about cirmtuzumab's potential for the treatment of patients with ROR1-expressing cancers, including MCL, CLL, HER2-negative breast cancer and other solid tumors, and look forward to further advancing its development to benefit patients with significant unmet medical needs."

MCL is an aggressive form of non-Hodgkin's lymphoma. MCL prevalence is estimated to be approximately 13,000 to 21,000 patients in the United States. MCL is an aggressive cancer that carries a poor prognosis, with a median survival of about two to five years and a 10-year survival rate of approximately 5%-10%.

CLL is the most common form of leukemia in adults, accounting for 25-30% of all leukemias in the United States. CLL prevalence is estimated to be approximately 158,000 to 178,000 patients in the U.S. Despite various recently approved therapies, CLL generally remains incurable.

### About Cirmtuzumab

Cirmtuzumab is an investigational, potentially first-in-class monoclonal antibody targeting ROR1, or Receptor tyrosine kinase-like Orphan Receptor 1. Cirmtuzumab is currently being evaluated in a Phase 1/2 clinical trial in combination with ibrutinib for the treatment of CLL or MCL, in a collaboration with the University of California San Diego School of Medicine and the California Institute for Regenerative Medicine (CIRM). In addition, an investigator-initiated Phase 1 clinical trial of cirmtuzumab in combination with paclitaxel for women with HER2-negative metastatic breast cancer is being conducted at the UC San Diego School of Medicine.

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. Preclinical data showed that when cirmtuzumab bound to ROR1, it blocked Wnt5a signaling, inhibited tumor cell proliferation, migration and survival, and induced differentiation of the tumor cells. The FDA has granted Orphan Drug Designations to cirmtuzumab for the treatment of mantle cell lymphoma and chronic lymphocytic leukemia/small lymphocytic lymphoma. Cirmtuzumab is in clinical development and 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 chronic lymphocytic leukemia (CLL) and mantle cell lymphoma (MCL) 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, and [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 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](http://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: the expected benefits associated with orphan drug designation; the

potential of cirmtuzumab to treat ROR1 expressing cancers, including MCL, CLL, Her2-negative breast cancer and other solid tumors, and benefit patients with unmet medical needs. Forward looking statements are subject to risks and uncertainties inherent in Oncternal's business, which include, but are not limited to: 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; 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, 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 cirmtuzumab and Oncternal's other product candidates, which could adversely impact the company's ability to complete clinical trials and obtain regulatory approval for such product candidates; 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 cirmtuzumab 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 and its other product development programs; the risk that the regulatory landscape that applies to the development program for cirmtuzumab and the company's other product candidates may change over time; the risk that the approval of one of our 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 our 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 operations after the fourth quarter of 2020 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, 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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