Oncternal to Present Interim Phase 1/2 Cirmtuzumab Data at ASCO

May 15, 2019

Oncternal Therapeutics to Present Interim Data from a Phase 1/2 Study of Cirmtuzumab in Combination with Ibrutinib at the 2019 American Society of Clinical Oncology Annual Meeting

SAN DIEGO, May 15, 2019 -- Oncternal Therapeutics, Inc., a clinical-stage biotechnology company focused on developing potential first-in-class therapeutic candidates for cancers with critical unmet medical need, today announced that it will present interim data from an ongoing Phase 1/2 study of its investigational monoclonal antibody, cirmtuzumab, in combination with ibrutinib in patients with chronic lymphocytic leukemia (CLL), as part of a poster session at the 2019 American Society of Clinical Oncology (ASCO) Annual Meeting being held May 31 to June 4, 2019. The study is being conducted in collaboration with the California Institute for Regenerative Medicine (CIRM) and University of California San Diego (UC San Diego) School of Medicine.

Presentation details:
Title: Phase 1/2 Trial of Cirmtuzumab and Ibrutinib: Planned Analysis of Phase 1 CLL Cohorts
Abstract Number: 7527
Poster Session: Hematologic Malignancies – Lymphoma and Chronic Lymphocytic Leukemia
Session Time: Monday, June 3, 8:00-11:00 a.m. CDT, Hall A
Presenting Author: Michael Y. Choi, M.D., UC San Diego Moores Cancer Center

Study CIRM-0001 is a Phase 1/2 clinical trial evaluating cirmtuzumab in combination with ibrutinib, and is actively enrolling patients with CLL and mantle cell lymphoma (MCL). Part 1 was a dose-finding arm designed to determine the recommended dosing regimen (RDR), Part 2 is an expansion cohort to confirm the RDR, and Part 3 will randomize patients with CLL to receive either ibrutinib alone or ibrutinib plus cirmtuzumab. There was a planned interim analysis of the dose finding component of Part 1, and the results included:

- 12 patients with CLL were treated with cirmtuzumab at doses from 2 to 16 mg/kg, plus ibrutinib 420 mg.
- All patients remained on study with periods of observation from 16 to 48 weeks.
- The overall response rate was 67% with 1 confirmed complete response (CR) with no morphologic evidence of CLL in the marrow, 1 clinical complete response, 6 partial responses, and 4 stable disease. No patient had progressive disease.
- The typical redistributive lymphocytosis seen with ibrutinib was blunted, with only a 50% mean rise in absolute lymphocyte count, rapidly returning to baseline.

About Cirmtuzumab

Cirmtuzumab is an investigational, potentially first-in-class anti-ROR1 monoclonal antibody. Cirmtuzumab is currently in a Phase 1/2 clinical trial in combination with ibrutinib for the treatment of CLL and MCL, in a collaboration with UC San Diego School of Medicine and CIRM. In addition, an investigator-initiated Phase 1 clinical trial of cirmtuzumab in combination with paclitaxel for women with metastatic breast cancer is being conducted at UC San Diego School of Medicine. CIRM has also provided funding to support the cirmtuzumab development program. Receptor tyrosine kinase-like Orphan Receptor 1 (ROR1) is a survival factor for many cancers. Tumor cells that express ROR1 have tumor-initiating features that are associated with a dedifferentiated oncogenic state. When expressed by hematologic malignancies such as CLL and MCL, ROR1 acts as a receptor for the tumor growth factor Wnt5a. Researchers at UC San Diego School of Medicine discovered that targeting a critical epitope on ROR1 was the key to specifically targeting ROR1 expressing tumors, and this finding led to the discovery of the potent and highly selective antitumor activity of cirmtuzumab observed in preclinical studies. ROR1 activates pathways that lead to increased cancer cell proliferation, invasiveness and drug resistance. Oncternal believes ROR1 is an attractive target for cancer therapy because it is an oncofetal antigen – a protein not normally expressed in adults, and confers a survival and fitness advantage when reactivated and expressed by tumor cells. Overexpression of ROR1 in tumors results in cancer cells becoming less differentiated, increasing their ability to self-renew and metastasize by increasing cell migration and the ability to initiate new tumors. Patients with tumors that overexpress ROR1 have poor prognoses, consistent with the increased cell migration, tumor initiation, and chemotherapy resistance observed in preclinical models. When cirmtuzumab binds to ROR1, it blocks Wnt5a signaling, induces differentiation of the tumor cells, and inhibits tumor cell proliferation, migration and survival.

About Oncternal Therapeutics

Oncternal Therapeutics is a clinical-stage biopharmaceutical company focused on developing a diverse pipeline of 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 progression. The pipeline includes cirmtuzumab, a monoclonal antibody that is designed to inhibit the ROR1 receptor that is being evaluated in a Phase 1/2 clinical trial in combination with ibrutinib for the treatment of CLL and MCL, and TK-216, a small-molecule that is designed to inhibit ETS-family oncoproteins, which is being evaluated in a Phase 1 clinical trial alone and in combination with vincristine as a treatment for Ewing sarcoma, a rare pediatric cancer. 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 belief that ROR1 is an attractive target for cancer therapy; Oncternal’s plans to present data at the ASCO Annual Meeting; the timing of enrollment of the Phase 1/2 clinical trial of cirmtuzumab in combination with ibrutinib; and Oncternal’s clinical plans for cirmtuzumab, TK-216 and its CAR-T product candidate. Forward looking statements are subject to risks and uncertainties, which include, but are not limited to: uncertainties associated with the clinical development of, 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; risks related to the inability of Oncternal to obtain sufficient additional capital to continue to advance the development of its product candidates and preclinical programs; and uncertainty regarding whether potential 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. 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.

About Oncternal Therapeutics

Oncternal Therapeutics is a clinical-stage oncology company developing novel, potential first-in-class therapeutic candidates for cancers with critical unmet medical need. The company is leveraging its scientific and development expertise, as well as academic collaborations, to rapidly advance its pipeline.

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