Oncternal Provides Business Update and Announces Second Quarter 2020 Financial Results

August 6, 2020

- 58% complete response rate reported in patients with relapsed/refractory mantle cell lymphoma (MCL) in ongoing Phase 1/2 clinical trial of cirmtuzumab with ibrutinib, and the regimen was well tolerated. Meeting requested with FDA to discuss registration pathway for cirmtuzumab for patients with MCL

-Enrollment strong in expansion cohort of Phase 1 clinical trial of TK216 for patients with relapsed/refractory Ewing sarcoma

- Management to host webcast today at 5:00 pm ET (2:00 pm PT)

SAN DIEGO--(BUSINESS WIRE)--Aug. 6, 2020-- Oncternal Therapeutics, Inc. (Nasdaq: ONCT), a clinical-stage biopharmaceutical company focused on the development of novel oncology therapies, today provided a business update and reported second quarter 2020 financial results.

“We are extremely pleased by the progress achieved in our development programs and the enthusiasm that we have received from the medical community,” said James Breitmeyer, M.D., Ph.D., President and CEO, Oncternal. “We are expecting additional data read-outs in our clinical trials in patients with MCL and Ewing sarcoma in the second half of this year, which we believe could be transformative for our company.”

Recent Highlights

- In May 2020, a data update from the ongoing Phase 1/2 clinical trial of cirmtuzumab in combination with ibrutinib was presented at the American Society of Clinical Oncology (ASCO) 2020 Virtual Annual Meeting, showing a 58% complete response (CR) rate and 83% overall best objective response rate (ORR) for patients with relapsed/refractory MCL. The one patient who relapsed following prior CD19 CAR-T therapy achieved a CR on this study. Of the four patients who relapsed following a prior regimen that included ibrutinib, all four responded on this study, with two CRs and two partial responses. The combination of cirmtuzumab plus ibrutinib has been well tolerated, with adverse events consistent with those reported for ibrutinib treatment alone. Cirmtuzumab is an investigational, potentially first-in-class humanized monoclonal antibody that binds with high affinity to a biologically important epitope on ROR1 (Receptor-tyrosine kinase-like Orphan Receptor 1).
- In June 2020, based on encouraging data presented at ASCO 2020, we announced an increased focus on our cirmtuzumab ROR1 antibody program for patients with MCL. We are amending the ongoing Phase 1/2 study clinical trial of cirmtuzumab with ibrutinib to increase enrollment of patients with MCL in the Phase 2 expansion cohort to at least 20 patients and to allow enrollment of patients with a broader range of prior ibrutinib treatments. We have requested a meeting with the U.S. Food and Drug Administration (FDA) to seek guidance on a potential accelerated approval pathway for cirmtuzumab plus ibrutinib in patients with relapsed/refractory MCL.
- In June 2020, we announced that the FDA granted the company orphan drug designations of cirmtuzumab for treatment of MCL and for treatment of chronic lymphocytic leukemia (CLL)/small lymphocytic lymphoma.
- In July 2020, we hosted a virtual scientific presentation on the current treatment landscape of MCL, along with a discussion of the cirmtuzumab MCL clinical dataset, with Dr. Michael Wang, professor of Lymphoma & Myeloma at The University of Texas MD Anderson Cancer Center.
- Enrollment has accelerated in the expansion cohort of our ongoing, open-label, multicenter Phase 1 clinical trial of TK216 for patients with relapsed/refractory Ewing sarcoma, despite the COVID-19 pandemic, following our announcement in April 2020 of an interim clinical data update, including deep responses in two patients. We now anticipate announcing clinical data for 12 to 16 patients dosed in this expansion cohort in the fourth quarter of 2020, above our previous guidance of data for 7 to 12 patients. TK216 is an investigational, potentially first-in-class targeted small-molecule inhibitor of the E26 transformation-specific (ETS) family of oncoproteins.
- In May and July 2020, we announced an aggregate of $11.2 million in gross proceeds from registered direct offerings priced at-the-market under Nasdaq rules.

Expected Upcoming Milestones

- TK216 (ETS inhibitor) program
  - Clinical data for 12 to 16 patients with Ewing sarcoma treated in the Phase 1 expansion cohort to be presented at a scientific conference – in the fourth quarter of 2020
  - Potential Investigational New Drug Application (IND)-supporting data in additional ETS-driven tumors to be available – in the second half of 2020
- Cirmtuzumab (ROR1 antibody) program
  - Clinical data update for patients with MCL treated with cirmtuzumab plus ibrutinib in the ongoing Phase 1/2 study to be presented at a scientific conference – in the fourth quarter of 2020

- Clinical data update for patients with CLL treated with cirmtuzumab plus ibrutinib in the ongoing Phase 1/2 study to be presented at a scientific conference – in the fourth quarter of 2020
- Clinical data update for patients with HER2-negative breast cancer in the ongoing Phase 1b study to be presented at a scientific conference – in the first half of 2021
- Potential IND-supporting data in additional ROR1 expressing tumors to be available – in the second half of 2020

- ROR1 CAR-T program
  - First-in-human dosing in China – in 2021

Second Quarter 2020 Financial Results

Our grant revenue was $0.6 million for the second quarter ended June 30, 2020. Our grant revenue is derived from a sub-award under a grant from CIRM to UC San Diego, which was awarded to advance our Phase 1/2 clinical trial evaluating cirmtuzumab in combination with ibrutinib for the treatment of patients with MCL or CLL.

Our total operating expenses for the second quarter ended June 30, 2020 were $6.2 million. Research and development expenses for the quarter totaled $3.8 million, and general and administrative expenses for the quarter totaled $2.3 million. Net loss for the second quarter was $5.5 million, or a loss of $0.34 per share, basic and diluted.

As of June 30, 2020, we had $16.6 million in cash and cash equivalents. In addition, on July 21, 2020, we completed a registered direct offering priced at-the-market under Nasdaq rules, with gross proceeds of $6.2 million, before deducting placement agent's fees and other estimated offering expenses. We believe our current funds will be sufficient to fund our operations into the second quarter of 2021. Following the offering, we had 19.9 million shares of common stock outstanding.

Management Webcast

As previously announced, Oncternal will host a webcast today, August 6, 2020, at 5:00 p.m. ET (2:00 p.m. PT). The live webcast will be available online and may be accessed from the "Investors" page of the company website at http://investor.oncternal.com/. A replay of the webcast will be available beginning approximately one hour after the conclusion of the call and will remain available for at least 30 days thereafter.

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, 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.

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 including, without limitation, Oncternal's belief that the data read-outs from its clinical trials could be transformative, the numbers of patients expected to be enrolled and anticipated dates for announcing results from our clinical trials, and its belief that our current funds will be sufficient to fund its operations into the second quarter of 2021. Forward looking statements are subject to risks and uncertainties inherent in Oncternal's business, which include, but are not limited to: 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, TK216 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, 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 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; 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.

Oncternal Therapeutics, Inc.

Condensed Consolidated Balance Sheets Data

(in thousands)

	June 30, 2020	**December 31, 2019**
	(Unaudited)	
Cash and cash equivalents	$ 16,617	$ 20,051
Total assets	18,899	21,744
Total liabilities	9,301	7,432
Accumulated deficit	(75,845)	(65,572)
Total stockholders' equity	9,598	14,312

Oncternal Therapeutics, Inc.

Condensed Consolidated Statements of Operations Data

(Unaudited; in thousands, except per share data)

	Three Months Ended June 30,		**Six Months Ended June 30,**	
	2020	**2019**	**2020**	**2019**
Grant revenue	$ 623	$ 674	$ 1,201	$ 1,144
Operating expenses:				
Research and development	3,815	2,587	6,510	4,483
In-process research and development	—	18,088	—	18,088
General and administrative	2,343	1,619	4,977	2,551
Total operating expenses	6,158	22,294	11,487	25,122
Loss from operations	(5,535)	(21,620)	(10,286)	(23,978)
Other income (expense):				
Change in fair value of warrant liability	—	(1,285)	—	(1,268)

Interest income	—	59	13	106
Total other income (expense)	—	(1,226)	13	(1,162)
Net loss	$ (5,535)	$ (22,846)	$ (10,273)	$ (25,140)
Net loss per share, basic and diluted	$ (0.34)	$ (3.38)	$ (0.65)	$ (4.81)
Weighted-average shares outstanding, basic and diluted	16,241	6,765	15,798	5,229

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