Oncternal Provides Business Update and Announces First Quarter 2020 Financial Results

May 7, 2020

- Deep clinical responses reported for two of seven patients with relapsed/refractory Ewing sarcoma treated with the recommended Phase 2 dose of TK216 in ongoing Phase 1 clinical trial

- 50% complete response rate reported in patients with relapsed/refractory MCL in ongoing Phase 1/2 clinical trial of cirmtuzumab with ibrutinib

- Management to host webcast today at 3:00 pm ET

SAN DIEGO--(BUSINESS WIRE)--May 7, 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 first quarter 2020 financial results.

"Despite the COVID-19 pandemic, we are pleased to see continuing progress for our promising oncology pipeline, as we recently reported deep clinical responses in 28.5% of patients with Ewing sarcoma treated with TK216 at the selected Phase 2 dose with or without vincristine, and a 50% complete response ("CR") rate in patients with Mantle Cell Lymphoma ("MCL") treated with cirmtuzumab plus ibrutinib," said James Breitmeyer, M.D., Ph.D., President and CEO, Oncternal. "Our team has worked closely with our clinical investigators to help ensure the safety of the patients and the clinical study staff, while preserving regulatory compliance and data quality. At this time, we do not expect delays to our previously articulated clinical data read-outs in 2020."

Recent Highlights

- In April 2020, we announced an interim clinical data update for our ongoing, open-label, multicenter Phase 1 clinical trial of TK216, a targeted investigational small-molecule inhibitor of the E26 transformation-specific ("ETS") family of oncoproteins, in patients with relapsed or refractory Ewing sarcoma. As of the data cut-off date of March 26, 2020, seven patients treated at the recommended Phase 2 dose ("RP2D") of TK216 (200 mg/m ^{2}/day for 14 days without or with vincristine) were evaluable for clinical responses. Two of the seven patients (28.5%) had achieved partial responses ("PR"), one with a surgical CR, and one with 90% shrinkage of target lesions. Both responding patients remain on treatment, including one who has no evidence of disease after more than twelve months on study. Two patients had stable disease and three had progressive disease, for an overall clinical benefit of 57% (4/7).
- In March 2020, we announced an interim clinical data update for cirmtuzumab, a monoclonal antibody targeting ROR1, or Receptor tyrosine kinase-like Orphan Receptor 1, in combination with ibrutinib in patients with relapsed/refractory MCL enrolled in our ongoing Phase 1/2 clinical trial. As of the data cut-off of March 6, 2020, we reported a 50% CR rate for the 12 evaluable patients and an 83% best objective response rate ("ORR", including CR or PR). Responses were determined by Cheson criteria, and one of the six patients with CR had a complete metabolic response by PET scan, with a bone marrow biopsy pending.
- In February 2020, we presented ROR1 CAR-T preclinical data at the ASCO-SITC Clinical Immuno-Oncology Symposium. ROR1 CAR-T cell therapy demonstrated expansion, persistence and anti-tumor activity in an animal model of ROR1-expressing human leukemia. This research is being conducted by our collaborators at the University of California San Diego (UC San Diego) under a grant from the California Institute of Regenerative Medicine ("CIRM").
- Oncternal's first priority during the unprecedented public health emergency caused by the COVID-19 pandemic has been protecting the safety of the patients in our clinical trials, along with the safety of the staff at clinical sites and that of our own employees, while ensuring regulatory compliance and data integrity. Enrollment of new patients into our studies has slowed down, as most other drug developers are reporting, but we have continued to enroll additional patients into the Ewing sarcoma study recently despite COVID-19 related constraints. As of today, very few patients have discontinued their participation in our clinical trials because of COVID-19 concerns, and we remain on track to meet our previously disclosed expected clinical data milestones for 2020.

Expected Upcoming Milestones

- TK216 program
  - Clinical data for 7-12 patients with Ewing sarcoma treated in the Phase 1 expansion cohort – in the second half of 2020
  - IND-enabling data in additional ETS-driven tumors – in the second half of 2020
- Cirmtuzumab program
  - Clinical data update for patients with MCL, including for 15 or more patients treated with cirmtuzumab plus ibrutinib in the ongoing Phase 1/2 study – in mid-2020
  - Clinical data update for patients with CLL, including 12-month follow-up for up to 34 patients treated with

cirmtuzumab plus ibrutinib in the ongoing Phase 1/2 study – in mid-2020
    - Clinical data update for patients with HER2-negative breast cancer in the ongoing Phase 1b study – in the second half of 2020
    - IND-enabling data in additional ROR1 expressing tumors – in the second half of 2020
- ROR1 CAR-T program
    - First-in-human dosing in China – in the first half of 2021

First Quarter 2020 Financial Results

Our grant revenue was $0.6 million for the first quarter ended March 31, 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 first quarter ended March 31, 2020 were $5.3 million. Research and development expenses for the quarter totaled $2.7 million, and general and administrative expenses for the quarter totaled $2.6 million. Net loss for the first quarter was $4.7 million, or a loss of $0.31 per share, basic and diluted.

As of March 31, 2020, we had $16.0 million in cash and cash equivalents. We believe these funds will be sufficient to fund our operations into the fourth quarter of 2020. As of March 31, 2020, we had approximately 15.4 million shares of common stock outstanding.

Management Webcast

As previously announced, Oncternal will host a webcast today, May 7, 2020, at 3:00 p.m. ET (12:00 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 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.

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 belief in the potential of its clinical product candidates to meet critical unmet medical needs; expectations regarding advancing its clinical programs in 2020 and the timing for the disclosure of additional data from the company's ongoing clinical trials of cirmtuzumab and TK216; the timing for first-in-human dosing in China for its ROR1 CAR-T product candidate; Oncternal's assessment of the impact of the COVID-19 pandemic on its business operations and clinical trials; and the company's belief that it has sufficient funds to fund its development programs and operations into the fourth quarter of 2020. The inclusion of forward-looking statements should not be regarded as a representation by Oncternal that any of its plans will be achieved. Actual results may differ from those set forth in this release due to the risks and uncertainties inherent in Oncternal's business, including, without limitation: Oncternal may encounter further 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; Oncternal's dependence on the success of cirmtuzumab, TK216 and its other product development programs; uncertainties associated with the clinical development and process for obtaining regulatory approval of cirmtuzumab, TK216 and Oncternal's other product candidates, including additional delays in enrollment and potential delays in the commencement and completion of clinical trials; 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; the risk that the regulatory landscape that applies to the development programs for the company's product candidates may change, which could result in delays or termination of development of such product candidates or unexpected costs in obtaining regulatory approvals; 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 Oncternal'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)

	March 31, 2020	December 31, 2019
Cash and cash equivalents	$ 16,019	$ 20,051
Total assets	17,765	21,744
Total liabilities	7,370	7,432
Accumulated deficit	(70,310)	(65,572)
Total stockholders' equity	10,395	14,312

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Condensed Consolidated Statements of Operations Data

(Unaudited; in thousands, except per share data)

	Three Months Ended March 31,	
	2020	2019
Grant revenue	$ 578	$ 470
Operating expenses:		
Research and development	2,696	1,896
General and administrative	2,633	932
Total operating expenses	5,329	2,828
Loss from operations	(4,751)	(2,358)
Other income:		
Change in fair value of warrant liability	—	17
Interest income	13	47
Total other income	13	64
Net loss	$ (4,738)	$ (2,294)
Net loss per share, basic and diluted	$ (0.31)	$ (0.62)
Weighted-average shares outstanding, basic and diluted	15,355	3,676

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