Oncternal Announces Third Quarter 2020 Financial Results and Provides Business Update

November 4, 2020

- ***Rare Pediatric Disease Designation received from FDA for TK216 for treatment of Ewing sarcoma***
- ***Interim Phase 1 data for TK216 in 15 evaluable patients with relapsed/refractory Ewing sarcoma demonstrated two complete responses, with no relapses after complete response***
- ***Strengthened the balance sheet by securing $10.1 million in equity financing during Q3 2020***

SAN DIEGO, Nov. 04, 2020 (GLOBE NEWSWIRE) -- 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 third quarter 2020 financial results.

“In the third quarter we continued advancing our oncology pipeline while strengthening our balance sheet,” said James Breitmeyer, M.D., Ph.D., President and CEO, Oncternal. “We expect to provide additional data updates on our clinical trials in patients with mantle cell lymphoma (MCL), Ewing sarcoma, and chronic lymphocytic leukemia (CLL) in the fourth quarter.”

Recent Highlights

- In July 2020, we hosted a virtual scientific presentation on the current treatment landscape of MCL, along with a discussion of our cirmtuzumab MCL clinical data set, with Dr. Michael Wang, professor of Lymphoma & Myeloma at The University of Texas MD Anderson Cancer Center. A data update from our ongoing open-label Phase 1/2 clinical trial of cirmtuzumab in combination with ibrutinib that was presented at the American Society of Clinical Oncology (ASCO) 2020 Virtual Annual Meeting in May 2020 showed a 58% complete response (CR) rate and 83% overall best objective response rate (ORR) for patients with relapsed/refractory MCL, as of the April 30, 2020 data cut-off date. 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 September 2020, we met with the U.S. Food and Drug Administration (FDA) and are in a dialogue with the FDA regarding potential accelerated approval pathways for cirmtuzumab plus ibrutinib in patients with relapsed/refractory MCL.
- In September 2020, a data update from the ongoing open-label Phase 1 clinical trial of TK216 in patients with relapsed or refractory Ewing sarcoma was given as an oral presentation at the European Society for Medical Oncology (ESMO) Virtual Congress 2020. The presentation included interim data for 15 evaluable patients treated at the recommended Phase 2 dose as of the August 13, 2020 data cut-off date. Two of the 15 patients had achieved complete responses (CR), including one surgical CR. Both CRs were ongoing and the patients remained on treatment. Five patients had stable disease (SD), for a disease control rate (CR, partial response or SD) of 47%. TK216 is an investigational, potentially first-in-class, targeted small-molecule inhibitor of the E26 transformation-specific (ETS) family of oncoproteins.
- In October 2020, we announced that the FDA granted Rare Pediatric Disease designation for TK216 for treatment of Ewing sarcoma.
- In July and September 2020, we raised an aggregate of $10.1 million in net proceeds from a registered direct offering and an underwritten offering.

Expected Upcoming Milestones

- TK216 (ETS inhibitor) program
    - Clinical data for over 16 patients with Ewing sarcoma treated in the ongoing expansion cohort to be presented at the Connective Tissue Oncology Society (CTOS) 2020 Virtual Annual Meeting on November 11, 2020
    - Preclinical data in additional ETS-driven tumors in the first half of 2021
- Cirmtuzumab (ROR1 antibody) programs
    - Clinical data update for patients with MCL and CLL treated with cirmtuzumab plus ibrutinib in the ongoing Phase 1/2 study to be presented at the American Society for Hematology (ASH) 2020 Virtual Annual Meeting on December 7, 2020
    - Clinical data update for patients with HER2-negative breast cancer in the ongoing Phase 1b study in the first half of 2021
    - Preclinical data in additional ROR1 expressing tumors in the first half of 2021
- ROR1 CAR-T program

- First-in-human dosing in China in 2021

Third Quarter 2020 Financial Results
Our grant revenue was $0.6 million for the third quarter ended September 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 third quarter ended September 30, 2020 were $5.0 million. Research and development expenses for the quarter totaled $3.0 million, and general and administrative expenses for the quarter totaled $1.9 million. Net loss for the third quarter was $4.4 million, or a loss of $0.22 per share, basic and diluted.

As of September 30, 2020, we had $21.3 million in cash and cash equivalents. We believe our current funds will be sufficient to fund our planned operations into the second quarter of 2021. As of September 30, 2020, we had 22.3 million shares of common stock outstanding.

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. The clinical pipeline also includes 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 utilizing the cirmtuzumab antibody backbone 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 the anticipated dates and venues for announcing additional preclinical and clinical data, Oncternal’s expectations concerning interactions with the FDA regarding potential accelerated approval pathways for cirmtuzumab plus ibrutinib in patients with relapsed/refractory MCL, and other statements regarding Oncternal’s development plans. 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 the ongoing clinical trials of cirmtuzumab and TK216 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; Oncternal has encountered delays, and may encounter additional delays or difficulties, in completing preclinical studies and enrolling and retaining 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, TK216 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, TK216 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 planned operations 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.

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Source: Oncternal Therapeutics, Inc.

Oncternal Therapeutics, Inc.
Condensed Consolidated Balance Sheets Data
(in thousands)

	September 30, 2020 (Unaudited)	December 31, 2019
Cash and cash equivalents	$ 21,259	$ 20,051
Total assets	23,552	21,744
Total liabilities	7,852	7,432
Accumulated deficit	(80,240)	(65,572)
Total stockholders' equity	15,700	14,312

Oncternal Therapeutics, Inc.
Condensed Consolidated Statements of Operations Data
(Unaudited; in thousands, except per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Grant revenue	$ 585	$ 544	$ 1,787	$ 1,689
Operating expenses:				
Research and development	3,047	3,108	9,558	7,591
In-process research and development	—	—	—	18,088
General and administrative	1,933	2,385	6,910	4,937
Total operating expenses	4,980	5,493	16,468	30,616
Loss from operations	(4,395)	(4,949)	(14,681)	(28,927)
Other income (expense):				
Change in fair value of warrant liability	—	—	—	(1,268)
Interest income	—	57	13	163
Total other income (expense)	—	57	13	(1,105)
Net loss	$ (4,395)	$ (4,892)	$ (14,668)	$ (30,032)
Net loss per share, basic and diluted	$ (0.22)	$ (0.32)	$ (0.85)	$ (3.48)
Weighted-average shares outstanding, basic and diluted	20,126	15,340	17,251	8,636

Source: Oncternal Therapeutics