Oncternal Therapeutics Provides Business Update and Announces Fourth Quarter and Full Year 2020 Financial Results

March 11, 2021

- *Interim Phase 1/2 results for cirmtuzumab with ibrutinib in MCL presented at ASH in December 2020 compare favorably to historical single-agent ibrutinib data (47% CR vs. 20% CR historical single-agent ibrutinib)*
- *Accelerating development of ROR1-targeting CAR-T cell therapies*
- *Two durable complete responses in patients with metastatic relapsed/refractory Ewing sarcoma treated with TK216 in ongoing Phase 1/2 clinical trial*
- *Appointed Edwina Baskin-Bey, M.D., as Acting Chief Medical Officer*
- *Management to host webcast today at 5:00 pm ET*

SAN DIEGO, March 11, 2021 (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 fourth quarter and full year 2020 financial results.

“In 2021, we are advancing a deep pipeline of differentiated oncology assets. We have now initiated early-stage work in ROR1-targeting immunotherapies, including CAR-T and CAR-NK cell therapies, while moving forward the later-stage clinical development of cirmtuzumab, an antibody targeting ROR1, in MCL, which has generated encouraging data in this difficult to treat cancer. At the same time, we continue to evaluate TK216, an ETS inhibitor which has generated promising results in Ewing sarcoma,” said James Breitmeyer, M.D., Ph.D., Oncternal’s President and CEO. “We have also strengthened our balance sheet by raising $125 million in 2020, which provides us with the runway to advance our promising programs into 2023. We have several key data read-outs pending in the second quarter this year.”

Recent Highlights

- In January 2021, we announced an agreement with Lentigen Technology, Inc., a wholly-owned subsidiary of Miltenyi Biotec B.V. & Co. KG, to manufacture lentiviral vectors to support Oncternal’s investigational ROR1-targeting CAR-T cell therapy program.
- In January 2021, we announced a research and development collaboration with Karolinska Institutet in Stockholm, Sweden, to advance novel ROR1-targeting CAR-T and CAR-NK cell therapies from the laboratory into the clinic.
- In December 2020, we announced an interim clinical data update from the ongoing Phase 1/2 clinical trial of cirmtuzumab, an investigational anti-ROR1 monoclonal antibody, in combination with ibrutinib in patients with mantle cell lymphoma (MCL) and chronic lymphocytic leukemia (CLL) at the American Society of Hematology 2020 Virtual Annual Meeting. Best objective response rate of 87%, including complete response (CR) rate of 47%, was reported for 15 evaluable patients with relapsed/refractory (r/r) MCL. Median progression-free survival (PFS) was not reached for patients with MCL, with the 95% confidence interval above 17.5 months, after a median follow-up of 12.1 months. The median PFS was not reached for patients with treatment-naïve CLL (n=19) after a median follow-up of 16.6 months, and median PFS was 29.5 months for patients with r/r CLL (n=30) after a median follow-up of 17.1 months. The combination of cirmtuzumab and ibrutinib was well tolerated in this trial.
- In November 2020, we announced an interim clinical data update from the ongoing Phase 1/2 clinical trial evaluating TK216, an investigational, potentially first-in-class, targeted small-molecule inhibitor of the E26 transformation-specific (ETS) family of oncoproteins, in patients with r/r Ewing sarcoma at the Connective Tissue Oncology Society 2020 Virtual Annual Meeting. The reported disease control rate (CR, partial response or stable disease) was 43%, including two patients with durable complete responses that were ongoing at over 1.5 years and 8 months on treatment.
- In October 2020, we announced that the U.S. Food and Drug Administration granted Rare Pediatric Disease designation for TK216 for the treatment of Ewing sarcoma.
- In November and December 2020, we raised an aggregate of approximately $109 million in gross proceeds from two underwritten offerings.

Expected Upcoming Milestones

- 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 in Q2 2021
  - Clinical data update for patients with HER2-negative breast cancer in the ongoing Phase 1b study in Q2 2021
  - Preclinical data in additional ROR1 expressing tumors in Q2 2021

- ROR1 CAR-T program
  - First-in-human dosing in China in the second half of 2021
- TK216 (ETS inhibitor) program
  - Clinical data for patients with Ewing sarcoma treated in the ongoing Phase 1/2 expansion cohort in Q2 2021
  - Preclinical data in additional ETS-driven tumors in Q2 2021

Fourth Quarter and Full Year 2020 Financial Results
Our grant revenue was $1.6 million for the fourth quarter ended December 31, 2020. Our grant revenue is derived from a subaward under a grant from the California Institute for Regenerative Medicine (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. For the full year 2020, grant revenue was $3.4 million.

Our total operating expenses for the fourth quarter ended December 31, 2020 were $4.4 million. Research and development expenses for the quarter totaled $3.0 million, and general and administrative expenses for the quarter totaled $1.5 million. Net loss for the fourth quarter was $2.6 million, or a loss of $0.09 per share, basic and diluted. For the full year 2020, total operating expenses were $20.9 million. Net loss for the full year 2020 was $17.2 million, or a loss of $0.85 per share, basic and diluted.

As of December 31, 2020, we had $116.7 million in cash and cash equivalents. We believe these funds will be sufficient to fund our operations into 2023. As of December 31, 2020, we had approximately 48.8 million shares of common stock outstanding.

Management Webcast
As previously announced, Oncternal will host a webcast today, March 11, 2020, at 5:00 p.m. ET. 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. 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 Oncternal's development programs, including the anticipated timing for announcing additional preclinical and clinical data; timing of reaching any milestones with respect to Cirmtuzumab, TK216 or Oncternal's CAR-T program; and Oncternal's expected cash runway. Forward looking statements are subject to risks and uncertainties inherent in Oncternal's business, which include, but are not limited to: Oncternal's anticipated expenses may be greater than currently anticipated; 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 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)

	December 31, 2020	December 31, 2019
Cash and cash equivalents	$ 116,737	$ 20,051
Total assets	118,809	21,744
Total liabilities	5,858	7,432
Accumulated deficit	(82,797)	(65,572)
Total stockholders' equity	112,951	14,312

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

	Three Months Ended December 31,		Years Ended December 31,	
	2020	2019	2020	2019
	(Unaudited)			
Grant revenue	$ 1,588	$ 737	$ 3,375	$ 2,425
Operating expenses:				
Research and development	2,986	2,568	12,544	10,159
In-process research and development	—	—	—	18,088
General and administrative	1,464	2,350	8,373	7,286
Total operating expenses	4,450	4,918	20,917	35,533
Loss from operations	(2,862)	(4,181)	(17,542)	(33,108)
Other income (expense):				
Change in fair value of warrant liability	—	—	—	(1,268)
Other income	301	—	301	—
Interest income	3	25	16	188
Total other income (expense)	304	25	317	(1,080)
Net loss	$ (2,558)	$ (4,156)	$ (17,225)	$ (34,188)
Net loss per share, basic and diluted	$ (0.09)	$ (0.27)	$ (0.85)	$ (3.31)
Weighted-average shares outstanding, basic and diluted	29,398	15,350	20,305	10,329

Source: Oncternal Therapeutics