Cortexyme Announces First Quarter 2020 Financial Results and Provides Business Update

May 12, 2020

—Interim analysis for the GAIN Trial on track for Q4 2020, with top-line results from study's final analysis expected in Q4 2021

—Cortexyme shared additional scientific evidence supporting the gingipain hypothesis via multiple presentations and publications

SOUTH SAN FRANCISCO, Calif.--(BUSINESS WIRE)--May 12, 2020-- Cortexyme, Inc. (Nasdaq: CRTX), a clinical stage biopharmaceutical company pioneering potential therapeutics for Alzheimer's and other degenerative diseases, today announced financial results for the first quarter 2020 and provided an update on its business.

"The current global environment is challenging on many levels, but Cortexyme continues to execute and progress toward our goal of developing a novel treatment to halt progression of Alzheimer's disease," said Casey Lynch, Cortexyme's chief executive officer, co-founder, and chair. "We remain highly focused on the execution of the GAIN Trial, which is currently on track to complete enrollment of 570 patients this year and for which top-line results are anticipated in the fourth quarter of 2021. At the same time, we continue to research and publish new data that sheds light on the potential clinical utility of gingipain inhibitors for treatment of Alzheimer's and other diseases. With a strong balance sheet and a talented team, we're committed to creating value for all of our stakeholders, especially the neurodegenerative disease patients and caregivers in need of new therapeutic options."

GAIN Trial Updates

- The GAIN Trial, Cortexyme's Phase 2/3 clinical trial of COR388 versus placebo in patients with mild to moderate Alzheimer's disease, remains on track to report top-line results from its final analysis in the fourth quarter of 2021.
- As announced in February 2020, following discussion with the FDA, Cortexyme intends to conduct an interim analysis for overwhelming efficacy in the GAIN Trial. This interim analysis for overwhelming efficacy is currently planned for the fourth quarter of 2020 and will be conducted after approximately 100 patients in each of the GAIN Trial's three arms reach six months of treatment. As with the study's final analysis, the co-primary endpoints for the GAIN Trial's interim analysis are change from baseline in ADAS-Cog11 and CDR-SB versus placebo.
- The GAIN Trial includes a periodontal sub-study in which sites with a dental sub-investigator are assessing efficacy endpoints against periodontal disease, including pocket depth, at baseline, six months, and one year. While periodontal disease is not a criterion for enrollment in the GAIN Trial or the sub-study, more than 90% of patients enrolled in the sub-study as of March 2020 had moderate to severe periodontal disease at baseline.
- The GAIN Trial also includes an open-label extension (OLE) in the United States that is currently dosing patients. Upon completing the 48-week placebo-controlled period of the GAIN Trial, participants in the GAIN Trial's placebo and active arms in the U.S. may be eligible to enroll in the OLE study, where they will receive 80 mg of COR388 twice daily for an additional 48 weeks. The OLE is intended to evaluate long-term safety and efficacy measures of participants in the GAIN Trial.

Scientific Updates: Advancing a New Understanding of Alzheimer's Causation and Treatment
As COR388 moves through late-stage clinical development, Cortexyme and external collaborators continue to present and publish new research to advance understanding of the gingipain hypothesis for Alzheimer's pathogenesis and the clinical potential of COR388. Recent scientific presentations and publications are as follows:

- In January 2020, Cortexyme scientists and collaborators published new data in *Pharmacology Research and Perspectives* revealing further detail about the pharmacodynamics and utility of COR388. COR388 was efficacious in improving downstream pathology of the infection, namely, gingival pocket depth, a symptom of periodontal disease which affects approximately 65 million Americans. In addition, gingipain antigens and *P. gulae* DNA were found in the brains of aged dogs, indicating that *P. gulae* can also migrate from the oral cavity to the brain in a manner similar to that seen for *P. gingivalis* in Alzheimer's patients.
- In March 2020, Cortexyme posted on its website two data sets intended for medical meetings that were impacted by the ongoing COVID-19 pandemic. The first data set, slated for the International Association for Dental Research annual meeting, demonstrates the activity of COR388 in multiple animal models of periodontal disease, including mice and naturally aged canines. The second data set, part of the American Chemical Society National Meeting and Expo, highlights COR388's potential to decrease fragmentation of ApoE in the Alzheimer's disease central nervous system.
- In April 2020, Cortexyme announced a presentation at the virtual AAT-AD/PD Advances in Alzheimer's and Parkinson's Therapies meeting demonstrating that *in vivo* infection of primary neurons by *P. gingivalis* results in an Alzheimer's-like phenotype including synaptic loss, ultrastructural changes, and inflammation.
- In May 2020, Cortexyme announced the planned June 2020 publication in *The Journal of Alzheimer's Disease* of research further documenting the ability of *P. gingivalis* to invade neurons and trigger Alzheimer's-like neuropathology . An early online version of the paper is available now so that the important findings can be rapidly shared with the research community.

Corporate Updates

- In addition to other ongoing pipeline screening activities, Cortexyme is currently screening its proprietary library of small molecules for a possible treatment for coronaviruses. The virus responsible for COVID-19, SARS-CoV2, expresses a cysteine protease known as Mpro or 3CLpro that is required for replication, and this cysteine protease has been previously validated as a therapeutic target for coronaviruses. An initial screen of representative compounds from Cortexyme's library for 3CLpro protease inhibition identified a family of small-molecule inhibitors. Follow-up studies are being conducted to determine activity and potency of these inhibitors in SARS-CoV2 viral replication screens.
- In May 2020, Caryn McDowell, J.D. joined Cortexyme's executive leadership team as Chief Legal and Administrative Officer and Corporate Secretary. In this newly created role, she leads Cortexyme's legal, corporate governance, compliance, human resources, and administrative functions. Ms. McDowell has over two decades of leadership in biopharmaceutical organizations, including C-level experience both at development and commercial stage public companies. She joined Cortexyme from Revance, Inc., and previously served in leadership roles at Cytokinetics and Intermune.

Financial Results for the Quarter Ended March 31, 2020

Cash, Cash Equivalents and Marketable Securities: Cash, cash equivalents, and short and long-term marketable securities as of March 31, 2020, were $223.5 million, and includes approximately $117.7 million of net proceeds raised in Cortexyme's successful private placement offering completed in February 2020. Cortexyme expects current cash, cash equivalents and marketable securities will be sufficient to fund its operating and capital expenditures through 2022 and the completion of the GAIN Trial.

Research and Development (R&D) Expenses: For the quarter ended March 31, 2020, R&D expenses were $14.4 million. The expense was primarily due to costs related to the research and development of COR388 and the GAIN Trial.

General and Administrative (G&A) Expenses: For the quarter ended March 31, 2020, G&A expenses were $3.5 million. The expense was primarily attributable to personnel-related expenses, insurance, professional and legal fees, and stock-based compensation.

Net Loss: For the quarter ended March 31, 2020, net loss was $17.2 million, or a loss of $0.61 per basic share.

About Cortexyme, Inc.

Cortexyme (Nasdaq: CRTX) is a clinical stage biopharmaceutical company pioneering a novel, disease-modifying therapeutic approach to treat what it believes to be a key underlying cause of Alzheimer's disease and other degenerative diseases. Cortexyme is targeting a specific, infectious pathogen found in the brain of Alzheimer's patients and tied to neurodegeneration and neuroinflammation in animal models. Cortexyme's lead investigational medicine, COR388, is the subject of the GAIN Trial, an ongoing Phase 2/3 clinical study in patients with mild to moderate Alzheimer's disease. To learn more about Cortexyme, visit www.cortexyme.com or follow @Cortexyme on Twitter.

Forward-Looking Statements

Statements in this press release contain "forward-looking statements" that are subject to substantial risks and uncertainties. Forward-looking statements contained in this press release may be identified by the use of words such as "anticipate," "expect," "believe," "will," "may," "should," "estimate," "project," "outlook," "forecast" or other similar words. Examples of forward-looking statements include, among others, statements we make regarding our business plans and prospects, cash forecasts, the timing and success of our clinical trials and related data, ability to fund planned operating and capital expenditures, the timing of announcements and updates relating to our clinical trials and related data, the timing of and our ability to enroll patients into our clinical trials, and the potential therapeutic benefits, safety and efficacy of our product candidate or library of compounds. Forward-looking statements are based on Cortexyme's current expectations and are subject to inherent uncertainties, risks and assumptions that are difficult to predict and could cause actual results to differ materially from what we expect. Further, certain forward-looking statements are based on assumptions as to future events that may not prove to be accurate. Factors that could cause actual results to differ include, but are not limited to, the risks and uncertainties described in the section titled "Risk Factors" in our Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC) on March 16, 2020, our Quarterly Report on Form 10-Q filed with the SEC on May 12, 2020, and other reports as filed with the SEC. Forward-looking statements contained in this press release are made as of this date, and Cortexyme undertakes no duty to update such information except as required under applicable law.

Cortexyme, Inc. Condensed Statements of Operations

(Unaudited)

(In thousands, except per share amounts)

	Three Months Ended March 31, 2020	**Three Months Ended March 31, 2019**
Operating expenses:		

Research and development	$ 14,380	$ 4,825
General and administrative	3,478	1,250
Total operating expenses	17,858	6,075
Loss from operations	(17,858)	(6,075)
Interest income	682	394
Net loss	(17,176)	(5,681)
Other comprehensive income/ (loss):		
Unrealized gain / (loss) on available for sales securities	(97)	26
Total comprehensive income/(loss)	$ (17,273)	$ (5,655)
Net loss per share - basic and diluted	(0.61)	(1.61)

Cortexyme, Inc. Condensed Balance Sheets

(Unaudited)

(In thousands, per share amounts)

	March 31, 2020	**December 31, 2019**
ASSETS		
Current assets:		
Cash and cash equivalents	$ 85,606	$ 51,214
Short term investments	76,810	48,650
Prepaid expenses and other current assets	6,172	6,192
Total current assets	168,588	106,056
Property and equipment, net	624	709
Operating lease right-of-use assets	637	625
Long term investments	61,139	16,763
Other assets	217	217

Total assets	$ 231,205	$ 124,370

LIABILITIES AND STOCKHOLDERS' EQUITY

Current liabilities:		
Accounts Payable	4,954	3,075
Accrued expenses and other current liabilities	8,153	5,817
Total current liabilities	13,107	8,892
Long-term operating lease liability	57	-
Total liabilities	13,164	8,892
Total stockholders' equity	218,041	115,478
Total liabilities and stockholders' equity	$ 231,205	$ 124,370

View source version on businesswire.com: https://www.businesswire.com/news/home/20200512005876/en/

Investor Contact:
Chris Lowe
Cortexyme, Inc.
Chief Financial Officer
clowe@cortexyme.com

Media Contact:
Hal Mackins
For Cortexyme, Inc.
hal@torchcomllc.com
(415) 994-0040

Source: Cortexyme, Inc.