Cortexyme Announces Second Quarter 2020 Financial Results and Provides Business Update

August 17, 2020

— GAIN Trial enrollment on track to complete in Q4 2020

— GAIN Trial interim analysis on schedule to complete by year end 2020

— Clinical pipeline expansion anticipated in 2021

SOUTH SAN FRANCISCO, Calif.--(BUSINESS WIRE)--Aug. 17, 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 second quarter 2020 and provided an update on its business.

"The participation of the medical community and patients in the GAIN study of atuzaginstat in mild to moderate Alzheimer's disease continues to be strong, and we're entering the final months of enrollment with key anticipated milestones on track," said Casey Lynch, Cortexyme's chief executive officer, co-founder, and chair. "We are looking forward to the GAIN interim analysis before the end of this year, and we anticipate top-line data from the trial in the fourth quarter of 2021. At the same time, our research efforts continue to be productive and we are advancing additional molecules into IND-enabling studies, with the goal of expanding and adding value to our clinical pipeline in 2021. With a strong balance sheet and a talented team, we believe we are well positioned to potentially provide new therapeutic options to patients stricken with Alzheimer's and other neurodegenerative diseases."

GAIN Trial Updates: Evaluating Atuzaginstat, a New Potential Therapy for Alzheimer's Disease

- The Phase 2/3 GAIN Trial of atuzaginstat (COR388) versus placebo in patients with mild to moderate Alzheimer's disease (AD) has surpassed 500 patients enrolled and remains on track to report top-line results from its final analysis in the fourth quarter of 2021.
- Cortexyme remains on track to conduct an interim analysis in the GAIN Trial before year end 2020. This interim analysis for overwhelming efficacy, futility and sample size adjustment will be conducted after approximately 100 patients in each of the GAIN Trial's three arms reach 24 weeks of treatment. 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 also includes an ongoing open-label extension (OLE) study in the United States. 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 40 mg or 80 mg of atuzaginstat 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.
- A research abstract on the design of the GAIN Trial and its baseline biomarkers has been accepted as an oral presentation at the 13th Clinical Trials on Alzheimer's Disease (CTAD) meeting, which will be held in a hybrid virtual/onsite format in Boston from November 4-7, 2020. The abstract, "GAIN Trial Update and Baseline Biomarkers," will be presented on November 6th.

Scientific Updates: **Generating New Evidence and Expanding Our Opportunities to Help Patients**

As atuzaginstat advances through late-stage clinical development, Cortexyme and external collaborators continue to present and publish new research and study data to advance the gingipain hypothesis for Alzheimer's pathogenesis and identify additional development opportunities. Recent scientific presentations and research accomplishments include:

- At the virtual Alzheimer's Association International Conference® 2020 (AAIC®) last month, Cortexyme presented three posters, including new preclinical data in collaboration with the Forsyth Institute demonstrating the role of the bacterium *P. gingivalis* in AD and cardiovascular disease, providing a potential explanation for why the two diseases often occur together. Cortexyme also shared data demonstrating the therapeutic potential of atuzaginstat in treating both diseases.
- The in-life portion of a human radiolabeled mass balance study for atuzaginstat was completed on schedule and met the goal of achieving mass balance recovery, completing an important step in a study required by regulatory authorities for the registration of a new drug.
- A novel lysine gingipain inhibitor from Cortexyme's library, COR588, has been selected to begin IND-enabling studies and is expected to enter the clinic in 2021. Cortexyme is advancing a portfolio of gingipain inhibitors toward the clinic with distinct target therapeutic product profiles and intellectual property.
- Cortexyme completed additional screening of its proprietary library of small molecules for a possible treatment for coronaviruses. The Company has identified inhibitors of the 3CL protease of SARS-CoV-2 that block viral replication in cells. Cortexyme is continuing to screen analogs for potency, selectivity, pharmacokinetics and other important properties to identify potential candidate molecules for further progression.

Financial Results for the Quarter Ended June 30, 2020

Cash, Cash Equivalents and Marketable Securities: Cash, cash equivalents, and short and long-term marketable securities as of June 30, 2020, were $210.6 million, and includes approximately $117.6 million of net proceeds raised in Cortexyme's 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 June 30, 2020, R&D expenses were $14.1 million, primarily due to costs related to the research and development of atuzaginstat and the GAIN Trial.

General and Administrative (G&A) Expenses: For the quarter ended June 30, 2020, G&A expenses were $4.2 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 June 30, 2020, net loss was $17.6 million, or a loss of $0.60 per basic share. Weighted average shares outstanding for the quarter ended June 30, 2020 was 29,442,915.

About Cortexyme, Inc.

Cortexyme, Inc. (Nasdaq: CRTX) is a clinical stage biopharmaceutical company pioneering upstream therapeutic approaches designed to improve the lives of patients diagnosed with Alzheimer's and other degenerative diseases. Based upon the evidence generated to date, Cortexyme is currently advancing its lead therapeutic candidate, atuzaginstat (COR388), in the GAIN Trial, an ongoing Phase 2/3 clinical trial in patients with mild to moderate Alzheimer's disease. Cortexyme is targeting a specific, infectious pathogen found in the brain of Alzheimer's patients and tied to neurodegeneration and neuroinflammation in animal models. 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, the translation to humans of pre-clinical data; the pre-clinical results for our product candidates, the timing and success of our clinical trials and related data, the potential of atuzaginstat to treat Alzheimer's disease and cardiovascular disease, our 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 August 14, 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 June 30,		Six Months Ended June 30,	
	2020	**2019**	**2020**	**2019**
Operating expenses:				
Research and development	$ 14,086	$ 7,109	$ 28,467	$ 11,934
General and administrative	4,185	2,466	7,662	3,716
Total operating expenses	18,271	9,575	36,129	15,650
Loss from operations	(18,271)	(9,575)	(36,129)	(15,650)
Interest income	659	513	1,341	907
Net loss	(17,612)	(9,062)	(34,788)	(14,743)
Other comprehensive income:				

Unrealized gain on available for sales securities	748	103	651	129
Total comprehensive loss	(16,864)	(8,959)	(34,137)	(14,614)
Net loss per share - basic and diluted	(0.60)	(0.57)	(1.21)	(1.52)

Cortexyme, Inc. Condensed Balance Sheets
(Unaudited)
(In thousands, per share amounts)

	June 30, 2020	December 31, 2019
ASSETS		
Current assets:		
Cash and cash equivalents	$ 66,156	$ 51,214
Short term investments	78,348	48,650
Restricted cash	—	—
Prepaid expenses and other current assets	7,104	6,192
Total current assets	151,608	106,056
Property and equipment, net	565	709
Operating lease right-of-use assets, net	686	625
Long term investments	66,107	16,763
Other assets	244	217
Total assets	$ 219,210	$ 124,370
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 5,091	$ 3,075
Accrued expenses and other current liabilities	8,602	5,817
Short-term lease liability	—	—
Total current liabilities	13,693	8,892
Long-term operating lease liability	50	—
Total liabilities	13,743	8,892

Total stockholders' equity	205,467	115,478
Total liabilities and stockholders' equity	$ 219,210	$ 124,370

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