Cortexyme Provides Regulatory Update for COR388 Development Program in Alzheimer's Disease

February 13, 2020

- Interim analysis in the Phase 2/3 GAIN Trial expected to occur in Q4 2020

SOUTH SAN FRANCISCO, Calif.--(BUSINESS WIRE)--Feb. 13, 2020-- Cortexyme, Inc. (Nasdaq: CRTX) today provided an update on its clinical development plans for COR388, the company's lead investigational medicine in development for mild to moderate Alzheimer's disease (AD). The company conducted a Type C meeting with the FDA gathering agreement and feedback on development plans through NDA, including the Phase 2/3 GAIN Trial statistical analysis plan, metabolite characterization, nonclinical studies, population pharmacokinetics, and drug-drug interaction studies.

Following discussion with the FDA, the company intends to conduct an interim analysis for overwhelming efficacy in the ongoing GAIN Trial. The analysis, currently planned before year-end 2020, will be conducted after approximately 100 patients in each of the GAIN Trial's three arms complete six months of treatment. The co-primary endpoints for the interim analysis will be change from baseline in ADAS-Cog11 and CDR-SB versus placebo.

"Cortexyme is pleased with the collaborative and productive dialogue with the FDA's Division of Neurology Products," said Michael Detke, M.D., Ph.D., Cortexyme's Chief Medical Officer. "With COR388, we are pursuing a differentiated mechanism of action that targets the gingipains, or toxic proteases, released by the bacterium *P. gingivalis*, which we believe infects the brain and causes Alzheimer's pathology. The body of preclinical and clinical evidence generated to date suggests COR388 could have a favorable impact on inflammation, neurodegeneration, bacterial load of *P. gingivalis*, and cognitive testing."

Cortexyme continues to expect the GAIN Trial's final analysis in the fourth quarter of 2021. The co-primary endpoints will be change from baseline in ADAS-Cog11 and CDR-SB versus placebo. The GAIN Trial is 90% powered to meet statistical significance on both primary endpoints with the planned 570 randomized patients. Additional exploratory endpoints include other cognitive, functional and clinical outcomes, safety and tolerability measures, and biomarkers relating to *P. gingivalis* infection, as well as traditional AD biomarkers and brain volume measurements. The study is stratified for both AD severity (mild versus moderate) and ApoE4 carriers versus non-carriers.

For more information about the GAIN Trial, visit www.GAINtrial.com.

About the GAIN Trial

The GAIN (GingipAIN Inhibitor for Treatment of Alzheimer's Disease) Trial is a randomized, double-blind, placebo-controlled Phase 2/3 trial evaluating the efficacy, safety, and tolerability of COR388, Cortexyme's investigational gingipain inhibitor, in patients with mild to moderate Alzheimer's disease. The GAIN Trial also includes a sub-study measuring the efficacy of COR388 on symptoms of periodontal disease. The GAIN Trial has been enrolling since the second quarter of 2019, with top-line results from the study's final analysis expected in the fourth quarter of 2021. For more information on the trial, visit www.gaintrial.com.

About Cortexyme

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. The company'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. 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. Forward-looking statements are based on Cortexyme's current expectations and are subject to inherent uncertainties, risks and assumptions that are difficult to predict. 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 the final prospectus related to Cortexyme's initial public offering filed with the Securities and Exchange Commission on May 9, 2019 and Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on November 12, 2019. 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.

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