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

February 15, 2021

- Double-blind phase of GAIN Trial to continue as planned, with top-line data expected Q4 2021

- Open-Label Extension of atuzaginstat to stop dosing and enrollment

SOUTH SAN FRANCISCO, Calif.--(BUSINESS WIRE)--Feb. 15, 2021-- Cortexyme, Inc. (NASDAQ: CRTX), a clinical-stage biopharmaceutical company focused on Alzheimer's and other degenerative diseases, received a letter from the U.S. Food and Drug Administration (FDA) stating that a partial clinical hold has been placed on atuzaginstat (COR388) impacting the open-label extension (OLE) phase of the company's ongoing Phase 2/3 study, the GAIN Trial. Under the hold, no new participants will be enrolled in the OLE and currently enrolled OLE participants will be discontinued. Participants in the fully enrolled (N=643) double-blind, placebo-controlled randomized phase of the GAIN Trial will continue to receive study drug at their assigned dose, with top-line results from the double-blind GAIN Trial in Q4 2021.

The partial clinical hold was initiated following the review of hepatic adverse events in the atuzaginstat trial by the FDA. These events have been reversible and without any known long-term adverse effects for the participants. Cortexyme will continue to collaborate with the FDA on the overall development program for atuzaginstat.

"Cortexyme's highest priority is the safety of study participants," said Casey Lynch, Cortexyme's chief executive officer, co-founder, and chair. "The ongoing double-blind GAIN Trial in mild to moderate Alzheimer's disease will provide a critical assessment of efficacy and safety in the treatment of this devastating disease."

About the GAIN Trial and its Open Label Extension

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 atuzaginstat (COR388), Cortexyme's investigational gingipain inhibitor, in patients with mild to moderate Alzheimer's disease. The GAIN Trial includes a sub-study measuring the efficacy of atuzaginstat on symptoms of periodontal disease including gingival pocket depth. Top-line results from the GAIN Trial's final analysis are expected in the fourth quarter of 2021. For more information on the trial, visit www.gaintrial.com.

The GAIN Trial protocol also includes an 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. are 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. As discussed above, Cortexyme has stopped enrollment and dosing in the OLE following the institution of a partial clinical hold.

About Cortexyme

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 and other organs and tied to degeneration and inflammation in humans and 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 the partial clinical hold and ongoing correspondence with the FDA, and its related impact on the timing and success of our clinical trials, including with respect to atuzaginstat, the double-blind, placebo-controlled randomized phase of the GAIN Trial and open-label extension phase; the timing of announcements and updates relating to our clinical trials and related data; the potential therapeutic benefits, safety and efficacy of our product candidate or library of compounds; statements about our ability to obtain, and the timing relating to, and regulatory submissions and approvals with respect to our drug product candidate. 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 November 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.

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