Cortexyme Announces GAIN Trial of Atuzaginstat in Alzheimer's Disease Has Exceeded Study Enrollment Target of 570 Patients

September 24, 2020

- Interim analysis of the Phase 2/3 GAIN Trial expected to occur before year-end 2020

SOUTH SAN FRANCISCO, Calif.--(BUSINESS WIRE)--Sep. 24, 2020-- Cortexyme, Inc. (Nasdaq: CRTX) today announced that its GAIN Trial for Alzheimer's disease (AD) has exceeded its enrollment target of 570 patients. GAIN is a randomized, double-blind, placebo-controlled Phase 2/3 trial of atuzaginstat (COR388), Cortexyme's lead investigational medicine, in patients with mild to moderate AD. The GAIN Trial's protocol includes an interim analysis on the study's co-primary cognitive and global endpoints after approximately 300 patients reach 24 weeks of treatment, and this interim analysis is expected to occur before year-end 2020. Top-line results of the GAIN Trial's final analysis, to be performed once all study subjects complete the one-year study, are expected in Q4 2021.

The GAIN Trial was designed to enroll patients with mild to moderate Alzheimer's disease, randomized 1:1:1 to receive atuzaginstat 40 mg twice a day, 80 mg twice a day a day, or placebo. GAIN Trial enrollment has already exceeded the enrollment targets in the three double-blind arms. Although new screening of patients for the double-blind arms has ended, eligible patients who have already screened for the study will continue to enroll over the next several weeks.

"The GAIN Trial is an important study of a potential new approach to addressing Alzheimer's. We are pleased the trial has reached this milestone on schedule, and we look forward to completing the trial's interim analysis before year-end 2020," said Michael Detke, M.D., Ph.D., Cortexyme's Chief Medical Officer. "The significant need for new therapeutic options for Alzheimer's patients grows every day, and we are encouraged by the body of evidence supporting atuzaginstat's mechanism of action, which is 'upstream' of other approaches."

Atuzaginstat targets the toxic proteases, or gingipains, produced by *P. gingivalis*, which have been discovered in greater than 90% of post-mortem brains of patients with AD and shown to produce Alzheimer's pathology in infected animals. *P. gingivalis* is best known as a keystone bacterium in the development of periodontal disease. The GAIN Trial includes a periodontal sub-study, in which approximately 40% of GAIN Trial participants are also assessed for endpoints of efficacy in periodontal disease. Of the sub-study participants enrolled to date, greater than 90% had moderate to severe periodontal disease at baseline.

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 atuzaginstat (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 including gingival pocket depth. 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, 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 timing and success of our clinical trials and related data including the outcome of the interim analysis, the potential of atuzaginstat to treat Alzheimer's 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.

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