



Cortexyme Announces GAIN Trial of Atuzaginstat in Alzheimer's Disease Has Reached Enrollment Milestone of 500 Patients

August 14, 2020

- Open label extension is currently active for GAIN Trial completers in the United States with robust conversion of eligible patients

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

SOUTH SAN FRANCISCO, Calif.--(BUSINESS WIRE)--Aug. 14, 2020-- Cortexyme, Inc. (Nasdaq: CRTX) today announced that enrollment in its GAIN Trial for Alzheimer's disease (AD) has reached 500 patients toward the study's previously announced enrollment target of 570 subjects. 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 functional endpoints after approximately 300 patients reach 24 weeks of treatment; 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.

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.

"We are gratified to see the continued high level of engagement of our clinical sites and study participants and their caregivers, especially during the last several months," said Michael Detke, M.D., Ph.D., Cortexyme's chief medical officer. "The fact that GAIN Trial timelines have not been impacted by the COVID-19 pandemic reflects the devastating impact of Alzheimer's disease, the significant need for new therapeutic options for patients, and the growing interest in atuzaginstat's mechanism of action, which is 'upstream' of other approaches. We expect to complete GAIN enrollment in the next few months and look forward to sharing study results when available."

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, 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 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.

View source version on businesswire.com: <https://www.businesswire.com/news/home/20200814005238/en/>

Media Contact:

Hal Mackins
For Cortexyme, Inc.

hal@torchcomllc.com
(415) 994-0040

Investor Contact:

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

Source: Cortexyme, Inc.