Vertex Provides Update on its Clinical Programs Targeting Alpha-1 Antitrypsin Deficiency

October 14, 2020

- Phase 2 study of VX-814 in patients with alpha-1 antitrypsin deficiency discontinued based upon safety and pharmacokinetic data -

- Phase 2 study of VX-864 continues to enroll and dose patients; data expected in H1 2021 -

- AATD research program continues to progress -

BOSTON--(BUSINESS WIRE)--Oct. 14, 2020-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today provided an update on its clinical programs targeting the small molecule correction of alpha-1 antitrypsin deficiency (AATD).

VX-814 Phase 2 Proof-of-Concept Study

Based on the safety and pharmacokinetic (PK) profile of VX-814 observed to date in a Phase 2 trial in AATD, Vertex has decided to stop dosing in the trial and will discontinue development of VX-814. The randomized, double-blind, placebo-controlled Phase 2 study of approximately 50 patients was designed to evaluate the safety and PK of VX-814, and the ability of VX-814 to increase functional levels of alpha-1 antitrypsin over 28 days of dosing. Elevated liver enzymes (AST/ALT) were observed in several patients. In four patients, across different doses studied, elevations greater than 8 times the upper limit of normal were noted; the elevated liver enzymes have either resolved or are resolving. No patients had concomitant elevation of bilirubin levels and all patients were asymptomatic. Analysis of the PK data from the study indicated that exposures achieved were low. Based on these data, Vertex concluded that it would not be feasible to safely reach targeted exposure levels, and thus meaningful increases in AAT levels, with VX-814, and the study was stopped.

“Based on the liver enzyme elevations observed, along with the determination that we would not be able to safely achieve targeted exposure levels with VX-814, we are discontinuing further development of this molecule,” said Carmen Bozic, M.D., Executive Vice President, Global Medicines Development and Medical Affairs, and Chief Medical Officer at Vertex. “We are grateful to the AATD patients and investigators who participated in the VX-814 studies and we remain committed to transforming the treatment of this disease. We look forward to continuing clinical study of VX-864 and other molecules targeting the underlying cause of AATD.”

VX-864 Phase 2 Proof-of-Concept Study

A Phase 2 trial of VX-864, which is structurally distinct from VX-814, was initiated in July 2020 and is ongoing. This randomized, double-blind, placebo-controlled study of approximately 40 patients is designed to evaluate the safety and pharmacokinetics of VX-864, and the ability of VX-864 to increase functional levels of alpha-1 antitrypsin over 28 days of dosing. Patients continue to enroll and be dosed in this study, and clinical data from this study are anticipated in the first half of 2021.

AATD Research Program

Vertex also continues to advance multiple small molecule correctors in late stage research with a goal of advancing at least one additional molecule into development in 2021.

About Vertex

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious diseases. The company has multiple approved medicines that treat the underlying cause of cystic fibrosis (CF) — a rare, life-threatening genetic disease — and has several ongoing clinical and research programs in CF. Beyond CF, Vertex has a robust pipeline of investigational small molecule medicines in other serious diseases where it has deep insight into causal human biology, including pain, alpha-1 antitrypsin deficiency and APOL1-mediated kidney diseases. In addition, Vertex has a rapidly expanding pipeline of genetic and cell therapies for diseases such as sickle cell disease, beta thalassemia, Duchenne muscular dystrophy and type 1 diabetes mellitus.

Founded in 1989 in Cambridge, Mass., Vertex's global headquarters is now located in Boston's Innovation District and its international headquarters is in London, UK. Additionally, the company has research and development sites and commercial offices in North America, Europe, Australia and Latin America. Vertex is consistently recognized as one of the industry's top places to work, including 10 consecutive years on Science magazine's Top Employers list and top five on the 2019 Best Employers for Diversity list by Forbes. For company updates and to learn more about Vertex's history of innovation, visit www.vrtx.com or follow us on Facebook, Twitter, LinkedIn, YouTube and Instagram.

Special Note Regarding Forward-looking Statements

This press release contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, including, without limitation, Dr. Bozic’s quote and statements regarding Vertex’s plans and expectations for the clinical trial evaluating VX-864 and Vertex’s AATD research program. While Vertex believes the forward-looking statements contained in this press release are accurate, these forward-looking statements represent the company's beliefs only as of the date of this press release and there are a number of factors that could cause actual events or results to differ materially from those indicated by such forward-looking statements. Those risks and uncertainties include, among other things, that data from the company's research and development programs may not support development or registration of its compounds due to safety, efficacy or other reasons, and other risks listed under Risk Factors in Vertex's annual report and quarterly reports filed with the Securities and Exchange Commission and available through the company's website at www.vrtx.com. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

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