



Vertex Initiates Phase 2 Clinical Trial Program for VX-548 for the Treatment of Acute Pain

July 19, 2021

- Proof-of-concept study of VX-548 in acute pain following bunionectomy surgery open for enrollment -

- Second Phase 2 study of VX-548 in acute pain following abdominoplasty surgery to begin in the coming weeks -

BOSTON--(BUSINESS WIRE)--Jul. 19, 2021-- [Vertex Pharmaceuticals Incorporated](#) (Nasdaq: VRTX) today announced that it has begun a Phase 2 proof-of-concept (POC) study in acute pain following bunionectomy surgery with the selective NaV1.8 inhibitor VX-548 and that it expects to commence a second Phase 2 study in acute pain following abdominoplasty surgery in the coming weeks.

"Based on the favorable Phase 1 data, we are advancing this program into Phase 2 with initiation of a dose-ranging POC study in patients with acute pain after bunionectomy surgery followed quickly by a second study in patients undergoing abdominoplasty surgery. We expect to see results from the bunionectomy study by Q1 2022," said Carmen Bozic, M.D., Executive Vice President, Global Medicines Development and Medical Affairs, and Chief Medical Officer at Vertex. "NaV1.8 is a genetically and pharmacologically validated target and we are excited about the potential for VX-548 as a new class of effective pain treatments without the limitations of current therapies, including the addictive potential of opioids."

The Phase 2 studies are randomized, double-blind, placebo-controlled trials that will evaluate multiple doses of VX-548 in patients with acute pain following bunionectomy surgery or abdominoplasty surgery. Both studies will also include a hydrocodone bitartrate /acetaminophen reference arm. The primary endpoint in both studies is the time-weighted Sum of the Pain Intensity Difference over the first 48 hours of treatment (SPID48).

About VX-548

VX-548 is an oral, selective NaV1.8 inhibitor that has completed Phase 1 studies in healthy volunteers. Based on a favorable pharmacokinetic, safety and tolerability profile, it was advanced to Phase 2 studies in 2021.

NaV1.8 is a voltage-gated sodium channel that plays a critical role in pain signaling in the peripheral nervous system. NaV1.8 is a genetically validated novel target for the treatment of pain, and Vertex has previously demonstrated clinical proof-of-concept with a small molecule investigational treatment targeting NaV1.8 in multiple pain indications including acute pain, neuropathic pain and musculoskeletal pain.

Vertex's approach is to selectively inhibit NaV1.8 using small molecules with the objective of creating a new class of medicines that have the potential to provide superior relief of acute pain without the limitations of opioids, including their addictive potential. VX-548 is the most recent molecule to enter clinical development from Vertex's portfolio of NaV1.8 inhibitors.

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 cell and genetic 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. 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 11 consecutive years on Science magazine's Top Employers list and a best place to work for LGBTQ equality by the Human Rights Campaign. 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, as amended, including, without limitation, statements by Dr. Carmen Bozic in this press release, and statements regarding our plans to begin a Phase 2 study evaluating VX-548 in acute pain following abdominoplasty surgery, the potential benefits of VX-548 as a treatment for acute pain, and our plans, expectations and anticipated timelines for these Phase 2 clinical studies, including our expectations for available data. 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 risks and uncertainties that could cause actual events or results to differ materially from those expressed or implied by such forward-looking statements. Those risks and uncertainties include, among other things, that data from a limited number of patients may not be indicative of final clinical trial results, that either or both of the studies may not be completed in the expected timeframe, or at all, that data from the company's development programs may not support registration or further development of its compounds due to safety, efficacy, or other reasons, and other risks listed under the heading "Risk Factors" in Vertex's most recent annual report and subsequent filings filed with the Securities and Exchange Commission at www.sec.gov and available through the company's website at www.vrtx.com. You should not place undue reliance on these statements. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

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