Vertex Announces NaV1.8 Inhibitor Advancing to Phase 2 Clinical Development

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- Proof-of-concept studies of VX-548 in acute pain to initiate in the second half of the year -

BOSTON--(BUSINESS WIRE)--Apr. 26, 2021-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that it will advance the selective NaV1.8 inhibitor, VX-548, into Phase 2 clinical development with clinical trials in patients with both visceral and non-visceral types of acute pain. The decision to move to Phase 2 was based on supportive Phase 1 data in healthy volunteers, including safety, tolerability and pharmacokinetics (PK). In these studies, the molecule exhibited a favorable profile at doses considerably lower than those required with our previous NaV1.8 inhibitors.

NaV1.8 is a voltage-gated sodium channel that plays a critical role in pain signaling in the peripheral nervous system. NaV1.8 is genetically validated as a 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.

"Vertex has previously validated the potential role of NaV1.8 inhibitors with positive proof-of-concept results in a variety of pain conditions," said Carmen Bozic, M.D., Executive Vice President, Global Medicines Development and Medical Affairs, and Chief Medical Officer at Vertex. "We are excited about VX-548, a potent NaV1.8 inhibitor, and we are moving forward to the Phase 2 proof-of-concept trials with urgency, as these studies are the key next step toward our ultimate goal of developing transformative medicines for the treatment of pain."

About VX-548

VX-548 is an oral, selective NaV1.8 inhibitor that has completed Phase 1 studies. Based on a favorable pharmacokinetic, safety and tolerability profile, it will advance to Phase 2 studies in the second half of 2021.

About the Planned Phase 2 Studies in Acute Pain

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

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 advance VX-548 into Phase 2 clinical studies in the second half of 2021, the potential benefits of VX-548, and our plans, expectations and anticipated timelines for these clinical trials. 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 data from the company's development programs may not support registration or further development of its compounds due to safety, efficacy, or other reasons, that the COVID-19 pandemic may impact the status or progress of our clinical trials, and other risks listed under the heading “Risk Factors” in Vertex's most recent annual report 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, or the scientific data presented. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

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Vertex Pharmaceuticals Incorporated
Investors:
Michael Partridge, +1 617-341-6108
or
Brenda Eustace, +1 617-341-6187
or
Manisha Pai, +1 617-429-6891

Media:
mediainfo@vrtx.com
or
U.S.: +1 617-341-6992
or
Heather Nichols: +1 617-839-3607
or
International: +44 20 3204 5275

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