



Vertex Announces Publication of VX-548 Positive Phase 2, Proof-of-Concept Results in New England Journal of Medicine

August 3, 2023

- First published manuscript of VX-548 preclinical and clinical trial results in a peer-reviewed journal -

BOSTON--(BUSINESS WIRE)--Aug. 3, 2023-- [Vertex Pharmaceuticals Incorporated](#) (Nasdaq: VRTX) today announced publication in the *New England Journal of Medicine* (NEJM) of preclinical data and the results from its Phase 2 proof-of-concept trials evaluating treatment with the selective, oral NaV1.8 inhibitor VX-548 for acute pain following abdominoplasty and bunionectomy surgeries.

"VX-548 preclinical studies demonstrated high selectivity and potency for the NaV1.8 sodium channel which translated into Phase 2 clinical results with strong efficacy, a favorable benefit-risk profile and promise in the treatment of acute pain," said Carmen Bozic, M.D., Executive Vice President, Global Medicines Development and Medical Affairs, and Chief Medical Officer at Vertex. "Our Phase 3 trials for VX-548 are well underway, and we look forward to the potential of bringing an effective, non-opioid therapeutic option to patients suffering from moderate to severe acute pain."

"Acute pain is frequently treated with opioids that come with significant safety and tolerability concerns, including sedation, respiratory depression and the potential for misuse and addiction," said Scott Weiner, M.D., M.P.H., co-author of the NEJM manuscript, Associate Professor of Emergency Medicine at Harvard Medical School and Attending Emergency Physician in the Department of Emergency Medicine at Brigham and Women's Hospital. "There has not been a new class of pain medicine in decades, and we urgently need new treatment options to help the millions of people affected by pain each year in the U.S. The Phase 2 results from VX-548 show the potential this medicine could bring to patients."

Vertex expects to complete the pivotal program in late 2023 and share results from these studies in late 2023 or early 2024. VX-548 has been granted Breakthrough Therapy and Fast Track designations in the U.S. for moderate to severe acute pain. In addition, Vertex has initiated a Phase 2 dose-ranging study of VX-548 in neuropathic pain.

About VX-548

VX-548 is an oral, selective NaV1.8 inhibitor that is highly selective for NaV1.8 relative to other NaV channels. 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 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 pain without the limitations of opioids, including their addictive potential. VX-548 is one of the most recent molecules 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 clinical pipeline of investigational small molecule, mRNA, cell and genetic therapies (including gene editing) in other serious diseases where it has deep insight into causal human biology, including sickle cell disease, beta thalassemia, APOL1-mediated kidney disease, acute and neuropathic pain, type 1 diabetes and alpha-1 antitrypsin deficiency.

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 13 consecutive years on Science magazine's Top Employers list and one of Fortune's 100 Best Companies to Work For. 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 Carmen Bozic, M.D., and Scott Weiner, M.D., M.P.H., in this press release,

and statements regarding the potential benefits of VX-548 as a treatment for patients with acute pain, expectations that the pivotal program will complete in late 2023, plans to share results from these studies in late 2023 or early 2024, and our expectations for VX-548. 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 the company's studies 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 data may not be available on the anticipated timeline, or at all, that our development programs may experience delays, 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 or the scientific data presented. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

(VRTX-GEN)

View source version on [businesswire.com](https://www.businesswire.com/news/home/20230803756665/en/): <https://www.businesswire.com/news/home/20230803756665/en/>

Vertex Pharmaceuticals Incorporated

Investors:

InvestorInfo@vrtx.com

or

Miroslava Minkova: +1 617-341-6135

Media:

mediainfo@vrtx.com

or

U.S.: 617-341-6992

or

Heather Nichols: +1 617-839-3607

or

International: +44 20 3204 5275

Source: Vertex Pharmaceuticals Incorporated