Vertex Announces Advancements of Suzetrigine (VX-548) in Acute and Neuropathic Pain

April 18, 2024

– Rolling submission for suzetrigine moderate-to-severe acute pain NDA granted by FDA; first module submitted and on track to complete filing this quarter –

– Successful completion of end-of-phase 2 FDA meeting for pain associated with diabetic peripheral neuropathy (DPN); Phase 3 program to initiate in 2H 2024 –

– Breakthrough Therapy designation for pain associated with DPN granted by FDA –

– Enrollment of Phase 2 study in patients with painful lumbosacral radiculopathy on track to complete by end of year –

BOSTON--(BUSINESS WIRE)--Apr. 18, 2024-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced important advancements across its suzetrigine pain program, which has the potential to be the first new class of medicine for acute and neuropathic pain in more than two decades. Suzetrigine is an oral selective NaV1.8 pain signal inhibitor (formerly known as VX-548).

Following the positive Phase 3 results in acute pain announced in January 2024, the Food and Drug Administration (FDA) has granted a rolling New Drug Application (NDA) submission for suzetrigine in moderate-to-severe acute pain. Vertex has started the rolling submission process and is on track to complete the submission in the second quarter of 2024. Suzetrigine was previously granted FDA Fast Track and Breakthrough Therapy designations in moderate-to-severe acute pain.

In neuropathic pain, Vertex released positive results from its Phase 2 study in December 2023 and recently completed a successful end-of-phase 2 meeting with the FDA. Vertex is now preparing to initiate a Phase 3 pivotal program of suzetrigine in patients with DPN in 2H 2024. In addition, the FDA recently granted suzetrigine Breakthrough Therapy designation for the treatment of pain associated with DPN.

The Phase 3 program will include two identical 12-week randomized, double-blind, placebo-controlled studies evaluating the efficacy and safety of suzetrigine (70 mg once daily) in patients with DPN. The primary endpoint for both studies will be the change from baseline in weekly average of daily pain intensity on the numeric pain rating scale (NPRS) assessed at Week 12 compared to placebo. Both studies will also include a key secondary endpoint of change from baseline in the weekly average of daily pain intensity on the NPRS at Week 12 compared to pregabalin. Approximately 1,100 patients are expected to enroll in each Phase 3 study. After completing participation in the randomized controlled studies, patients may roll over into an open-label study to evaluate the long-term safety and effectiveness of suzetrigine in DPN.

Additionally, Vertex continues to enroll its Phase 2 study of suzetrigine in patients with lumbosacral radiculopathy, or LSR, which is pain caused by impairment or injury to nerve roots in the area of the lumbar spine. The company is on track to complete enrollment in the Phase 2 LSR study by the end of the year.

“Today marks a significant milestone on our journey to redefine the treatment of pain,” said Carmen Bozic, M.D., Executive Vice President, Global Medicines Development and Medical Affairs, and Chief Medical Officer at Vertex. “Given the favorable benefit/risk profile demonstrated by suzetrigine across the entire clinical program and the positive interactions with regulators, we are excited by the opportunity to rapidly advance suzetrigine, a new non-opioid potential treatment, for the millions of patients suffering from acute and peripheral neuropathic pain.”

Next Steps for the Pain Portfolio

In line with its pain portfolio serial innovation strategy, Vertex continues to advance preclinical and clinical development of additional NaV1.8 and NaV1.7 pain signal inhibitors, for use alone or in combination, in acute and neuropathic pain. The company intends to advance its next generation NaV1.8 pain signal inhibitor VX-993 oral formulation into Phase 2 acute pain and peripheral neuropathic pain studies later this year. Vertex also anticipates initiating a Phase 1 study of an intravenous formulation of VX-993 later this year.

About Acute Pain

Acute pain is a disabling condition and is defined as pain lasting less than 3 months. It is estimated that over 80 million people are

prescribed a medicine for acute pain every year in the U.S. Due to limited treatment options, there is an unmet need in acute pain management to improve the patient experience and reduce the economic and societal burden.

About Peripheral Neuropathic Pain

Peripheral neuropathic pain, or PNP, is a significant area of unmet need for patients suffering from pain. PNP is a collection of chronic conditions including pain associated with diabetic peripheral neuropathy (DPN), lumbosacral radiculopathy (LSR), small fiber neuropathy and trigeminal neuralgia. DPN and LSR are two of the largest segments within the estimated 10 million patients who are prescribed a medicine for PNP every year in the U.S.

About Suzetrigine (VX-548)

Suzetrigine (formerly VX-548) is an investigational oral, selective NaV1.8 pain signal 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 suzetrigine has demonstrated a favorable benefit/risk profile in three Phase 3 studies and two Phase 2 studies in moderate-to-severe acute pain. Suzetrigine also demonstrated positive results and a well-tolerated profile in a Phase 2 study in patients with pain associated with diabetic peripheral neuropathy, a type of peripheral neuropathic pain. Vertex's approach is to selectively inhibit NaV1.8 using small molecules with the objective of creating a new class of pain signal inhibitors that have the potential to provide effective relief of pain without the limitations of currently available therapies, including the addictive potential of opioids.

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 approved medicines that treat the underlying causes of multiple chronic, life-shortening genetic diseases — cystic fibrosis, sickle cell disease and transfusion-dependent beta thalassemia — and continues to advance clinical and research programs in these diseases. Vertex also has a robust clinical pipeline of investigational therapies across a range of modalities in other serious diseases where it has deep insight into causal human biology, including acute and neuropathic pain, APOL1-mediated kidney disease, autosomal dominant polycystic kidney disease, type 1 diabetes, myotonic dystrophy type 1 and alpha-1 antitrypsin deficiency.

Vertex was founded in 1989 and has its global headquarters in Boston, with international headquarters in London. Additionally, the company has research and development sites and commercial offices in North America, Europe, Australia, Latin America and the Middle East. Vertex is consistently recognized as one of the industry's top places to work, including 14 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 LinkedIn, Facebook, Instagram, YouTube and Twitter/X.

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, statements made by Carmen Bozic, M.D., in this press release, statements regarding Vertex's expectations for the rolling submission to the FDA for suzetrigine in acute pain, including expectations to complete the submission in the second quarter of 2024, expectations to initiate the Phase 3 program of suzetrigine in patients with DPN in the second half of 2024, plans for the design of and enrollment expectations for the Phase 3 DPN program, plans to evaluate the long-term efficacy and safety of suzetrigine in patients with DPN, including enrollment expectations for this open-label study, and enrollment expectations for the Phase 2 study in LSR, including plans to complete enrollment in this study by the end of the year, our expectations for the anticipated benefits of suzetrigine, our plans to continue advancing additional NaV1.8 and NaV1.7 pain signal inhibitors in acute and neuropathic pain, our plans to advance VX-993 oral formulation into Phase 2 acute pain and PNP studies later this year, and our plans to initiate a Phase 1 study of an intravenous formulation of VX-993 later this year. 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 our clinical studies for suzetrigine may be delayed, and that data from the company's research and development programs may not support registration or further development of its compounds due to safety, efficacy, and other risks listed under the heading "Risk Factors" in Vertex's annual report and in subsequent filings filed with the Securities and Exchange Commission and available through the company's website at www.vrtx.com and www.sec.gov. 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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