Vertex Announces FDA Acceptance of New Drug Application for Suzetrigine for the Treatment of Moderate-to-Severe Acute Pain

July 30, 2024

– FDA grants priority review and assigns a Prescription Drug User Fee Act (PDUFA) target action date of January 30, 2025 –

– Suzetrigine, an investigational non-opioid pain signal inhibitor, has the potential to treat millions of patients who suffer from moderate-to-severe acute pain each year –

BOSTON--(BUSINESS WIRE)--Jul. 30, 2024-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that the U.S. Food and Drug Administration (FDA) has accepted its New Drug Application (NDA) submission for suzetrigine, an investigational, oral, selective NaV1.8 pain signal inhibitor to treat moderate-to-severe acute pain. Suzetrigine has the potential to be the first new class of medicine to treat acute pain in over twenty years.

The FDA has granted suzetrigine priority review and assigned a Prescription Drug User Fee Act (PDUFA) target action date of January 30, 2025. Suzetrigine has already been granted FDA Fast Track and Breakthrough Therapy designations for the treatment of moderate-to-severe acute pain.

“Today’s FDA filing acceptance for suzetrigine marks a critical milestone toward bringing this new, transformative non-opioid analgesic to the millions of patients suffering from moderate-to-severe acute pain each year in the U.S.,” said Nia Tatsis, Ph.D., Executive Vice President, Chief Regulatory and Quality Officer at Vertex. “The FDA’s granting of a priority review further reinforces the high unmet need in treating acute pain, and the filing brings us one step closer to our objective of filling the gap between medicines with good tolerability but limited efficacy and opioid medicines with therapeutic efficacy but known risks, including addictive potential.”

"In my 24 years practicing medicine, I have seen firsthand the desperate need for new non-opioid therapies for treating pain. Too many people today are either undertreated, dealing with negative side effects of currently available therapies or foregoing pain medications altogether for fear of becoming dependent on opioids,” said Scott Weiner, M.D., M.P.H., Vertex Acute Pain Steering Committee Chair, 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. “Prescribers and patients deserve new options."

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 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 is selectively expressed in peripheral pain-sensing neurons (nociceptors), where its role is to transmit pain signals (action potentials). 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 patients with 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 chronic 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, IgA nephropathy, 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, as amended, including, without limitation, the statements by Nia Tatsis, Ph.D., and Scott Weiner, M.D., M.P.H., in this press release, and statements regarding our expectations for the benefits of and potential for suzetrigine as a treatment for moderate-to-severe acute pain. While we believe 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 development programs may not support registration or further development of its compounds due to safety, efficacy, and other reasons, that future competitive or other market factors may adversely affect the commercial potential for suzetrigine, and other risks listed under the heading “Risk Factors” in Vertex's most recent annual report and subsequent quarterly reports 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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Vertex Pharmaceuticals Incorporated
Investors:
InvestorInfo@vrtx.com
Susie Lisa, CFA: +1 617-341-6108
or
Miroslava Minkova: +1 617-341-6135

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

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