Vertex Provides Pipeline and Business Updates in Advance of Upcoming Investor Meetings

January 12, 2025

BOSTON--(BUSINESS WIRE)--Jan. 12, 2025-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced multiple program updates ahead of upcoming investor meetings in January, including the company's scheduled webcast from the 43rd Annual J.P. Morgan Healthcare Conference on Monday, January 13, 2025, at 10:30 a.m. ET/7:30 a.m. PT.

"2024 marked another year of excellent progress for Vertex, as we reached more people with CF than ever before, began a new era of commercial diversification, and advanced and broadened our clinical stage pipeline," said Reshma Kewalramani, M.D., Chief Executive Officer and President of Vertex. "In 2025, we are poised to diversify our business further across multiple dimensions: our revenue, as we continue the launches of CASGEVY, ALYFTREK and potentially launch suzetrigine in acute pain; our pipeline, as we progress four potentially transformative medicines through pivotal trials; and our geographic footprint, as we expand both our commercial and clinical presence globally."

Disease Areas with Approved Medicines

Cystic Fibrosis (CF)

- **ALYFTREK (vanzacaftor/tezacaftor/deutivacaftor) approved in the U.S.**: On December 20, 2024, Vertex secured FDA approval for ALYFTREK, the once-daily next-in-class combination CFTR modulator for the treatment of people with CF 6 years and older who have at least one F508del mutation or another mutation in the CFTR gene that is responsive to ALYFTREK, which includes a total of 303 mutations. Global regulatory submissions for ALYFTREK, including in the U.K. and Europe, are currently under review.
- **TRIKAFTA:** Also on December 20, 2024, Vertex received FDA approval for the expanded use of TRIKAFTA in patients with 94 additional non-F508del CFTR mutations. With this approval, approximately 300 people in the U.S. are newly eligible for a medicine that treats the underlying cause of their disease. TRIKAFTA is now approved for patients with a total of 272 CFTR mutations.
- **VX-522:** The multiple ascending dose (MAD) portion of the Phase 1/2 study of VX-522 is underway, with data expected in the first half of 2025. VX-522 is a CFTR mRNA therapeutic that Vertex is developing in collaboration with Moderna for the more than 5,000 people with CF who cannot benefit from CFTR modulators.
- **Epidemiology and market opportunity update:** Vertex increased its estimates for the number of people with cystic fibrosis in the U.S., Europe, Australia, and Canada from approximately 92,000 to approximately 94,000. Additionally, Vertex continues to secure formal reimbursement for eligible patients in multiple countries that collectively comprise approximately 15,000 additional patients, of whom approximately 10,000 are eligible for treatment with CFTR modulators. Vertex previously served many of these markets through named patient sales.

Sickle Cell Disease (SCD) and Transfusion-Dependent Beta Thalassemia (TDT) – CASGEVY

- As of the end of 2024, Vertex has activated more than 50 authorized treatment centers (ATCs) globally and more than 50 patients have initiated cell collection.
- On December 31, 2024, Vertex received regulatory approval for CASGEVY in the United Arab Emirates (UAE) for the treatment of both SCD and TDT.
- In the U.S., Vertex recently negotiated a first-of-its-kind, voluntary agreement with the Centers for Medicare & Medicaid Services (CMS), which will provide a single outcomes-based arrangement for CASGEVY, available to all state Medicaid programs to ensure broad and equitable access for patients.

Pipeline Disease Areas

Acute Pain

- **Suzetrigine:** The FDA has assigned a PDUFA target action date of January 30, 2025, for suzetrigine for the treatment of moderate-to-severe acute pain. Suzetrigine was granted Priority Review by the FDA.
- The Non-Opioids Prevent Addiction In the Nation (NOPAIN) Act became effective on January 1st, 2025. The NOPAIN Act mandates that Medicare provide a separate add-on payment in the hospital outpatient or surgical center setting for FDA-approved non-opioid treatments for pain. Vertex expects suzetrigine in acute pain to be included on the list of treatments that qualify for add-on payment under this act, following potential suzetrigine FDA approval.
- Seven states have recently enacted legislation into law for the retail setting, specifying that opioids are not preferred over non-opioid therapies for the treatment of pain.

Peripheral Neuropathic Pain (PNP)

- **Suzetrigine:** Vertex continues to enroll and dose patients with diabetic peripheral neuropathy (DPN) in a Phase 3 pivotal trial of suzetrigine.
- Following the December 2024 release of Phase 2 results with suzetrigine in painful lumbosacral radiculopathy (LSR), a form of peripheral neuropathic pain, Vertex plans to advance suzetrigine into pivotal development for painful LSR, pending discussions with regulators on the study design and regulatory package.

IgA Nephropathy (IgAN) and other B Cell-Mediated Diseases

- The global Phase 3 RAINIER study of povetacicept is enrolling and dosing patients with IgAN in the U.S., Europe and Asia. Vertex expects to complete enrollment in the interim analysis cohort in 2025 for potential accelerated approval in the U.S., once this cohort reaches 36 weeks of treatment.
- Vertex has entered into an exclusive collaboration and license agreement with Zai Lab for the development and commercialization of povetacicept in mainland China, Hong Kong, Macau, Taiwan, and Singapore. Zai Lab will help advance clinical trials and make regulatory submissions in the licensed territory, and they will also be responsible for all commercialization activities in the licensed territory upon potential approval of povetacicept.

APOL1-Mediated Kidney Disease (AMKD) – Inaxaplin (VX-147)

- Vertex continues to enroll and dose patients with primary AMKD in the Phase 3 portion of the AMPLITUDE global Phase 2/3 pivotal clinical trial of inaxaplin, in which a 45 mg once-daily dose of inaxaplin is compared to placebo, on top of standard of care. Vertex expects to complete enrollment in the interim analysis cohort in 2025 for potential accelerated approval in the U.S., once this cohort reaches 48 weeks of treatment.
- Vertex plans to initiate AMPLIFIED, a Phase 2b open-label study of inaxaplin in patients with AMKD and diabetes or other co-morbidities currently not eligible for the AMPLITUDE Phase 2/3 pivotal trial, expanding the estimated potentially eligible population from 150,000 to 250,000 patients.

Type 1 Diabetes (T1D)

- **Zimislecel (VX-880):** Following successful end of Phase 2 meetings with the FDA, the European Medicines Agency (EMA), and the U.K. Medicines and Healthcare products Regulatory Agency (MHRA), Vertex initiated the Phase 3 portion of the Phase 1/2/3 study of zimislecel in patients with T1D with severe hypoglycemic events and impaired awareness of hypoglycemia. Vertex expects to complete enrollment and dosing of the pivotal study in 2025.
- **Epidemiology update:** Vertex estimates that a total of 125,000 patients have severe T1D, out of the estimated 3.8M people with T1D in North American and Europe. Vertex expects the initial zimislecel indication will address approximately 60,000 patients and is working to serve all 125,000 patients with severe diabetes over time.
- Consistent with its commitment to serial innovation and bringing transformative therapies to all patients who can benefit, Vertex is developing additional therapies for T1D that use the same cells that are used in zimislecel. This includes VX-264, currently in a Phase 1/2 study, in which the cells are encapsulated in an immunoprotective device. Vertex plans to share Part B full-dose data from the VX-264 Phase 1/2 study in 2025. Vertex is also pursuing alternative approaches to immunosuppression that could be used with zimislecel, as well as a hypoimmune program utilizing gene-edited stem-cell derived islets.

Myotonic Dystrophy Type 1 (DM1) – VX-670

- Vertex has completed the single ascending dose (SAD) portion of the global Phase 1/2 clinical trial for VX-670 in people with DM1 and initiated the MAD portion of the Phase 1/2 study, which will assess both safety and efficacy.

Autosomal Dominant Polycystic Kidney Disease (ADPKD) – VX-407

- Vertex is enrolling and dosing a Phase 1 study of healthy volunteers with VX-407. Vertex expects to advance VX-407 into a Phase 2 proof of concept study in people with ADPKD in 2025.

J.P. Morgan Healthcare Conference Presentation and Webcast

Dr. Kewalramani will present at the 43rd Annual J.P. Morgan Healthcare Conference on Monday, January 13, 2025, at 10:30 a.m. ET/7:30 a.m. PT.

A live webcast of management's remarks will be available through the Vertex website, www.vrtx.com, in the "Investors" section under the "News and Events" page. A replay of the conference webcast will be archived on the company's website.

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, primary membranous nephropathy, autosomal

dominant polycystic kidney disease, type 1 diabetes and myotonic dystrophy type 1.

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, statements by Reshma Kewalramani, M.D., and statements about our expectations for our CF program, including with respect to the diversification of Vertex's business and expanding commercially and clinically across more geographies, commercial expectations for ALYFTREK, the expectation to have data from the Phase 1/2 study of VX-522, expectations that VX-522 may treat >5,000 people with CF, the company's beliefs regarding CF epidemiology and market opportunities, expectations for the company's agreement with CMS and resulting patient access to CASGEVY, expectations that suzetrigine in acute pain will be included on the list of treatments that qualify for add-on payments under the NOPAIN Act, plans to advance suzetrigine into pivotal development for painful LSR, expectations regarding povetacicept in IgAN, including completing enrollment in the interim analysis cohort in 2025 for potential accelerated approval, expectations for the collaboration with Zai Lab, including the future activities of the parties pursuant to the collaboration, expectations regarding inaxaplin in AMKD, including that the company will complete enrollment in the interim analysis cohort in 2025 for potential accelerated approval in the U.S., plans to initiate a Phase 2b open-label study of inaxaplin in patients with AMKD and diabetes or other co-morbidities and expanding the eligible patient population, expectations regarding completion of enrollment and dosing in the pivotal study evaluating zimislecel in 2025, expectations regarding the initial eligible patient population that will benefit from zimislecel, plans to work with urgency to advance zimislecel to be able to serve all patients with severe T1D, plans to develop additional therapies for T1D, plans to share data from the VX-264 Phase 1/2 study in 2025, plans to pursue alternative approaches to immunosuppression that could be used with zimislecel and other T1D product candidates, and expectations to advance VX-407 into a Phase 2 proof of concept study in people with ADPKD in 2025. 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 clinical trial data might not be available on the expected timeline, that the anticipated benefits and potential of Vertex's collaboration with Zai Lab may not be achieved on the anticipated timeline, or at all, 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, that our discussions with regulators may be delayed or cause delays in our pipeline programs, 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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Source: Vertex Pharmaceuticals Incorporated