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

January 11, 2026

BOSTON--(BUSINESS WIRE)--Jan. 11, 2026-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced business and program updates ahead of upcoming investor meetings in January, including the company's scheduled webcast from the 44th annual J.P. Morgan Healthcare Conference on Monday, January 12, 2026, at 5:15 p.m. ET/2:15 p.m. PT.

"2025 was a year of strong commercial execution and rapid R&D progress, setting up the company for continued growth and many important milestones in 2026," said Reshma Kewalramani, M.D., Chief Executive Officer and President of Vertex. "Building on this momentum, we are focused on expanding our commercial reach in multiple disease areas; advancing the emerging renal franchise, including the potential near-term launch of povetacicept; and progressing our mid- and late-stage clinical pipeline. Vertex is well positioned to serve many more patients with our expanding portfolio of transformative medicines, and in so doing, deliver sustained growth and long-term value for shareholders."

Disease Areas with Approved Medicines

Cystic Fibrosis (CF)

- **ALYFTREK®:** ALYFTREK is now approved in the U.S., the United Kingdom (U.K.), the European Union (EU), Canada, New Zealand, Switzerland, and Australia for people with CF 6 years and older who have at least one F508del mutation or another mutation in the *cystic fibrosis transmembrane conductance regulator (CFTR)* gene that is responsive to ALYFTREK. Eligible patients in the U.S., England, Ireland, Germany, Denmark, Northern Ireland, Norway, and Wales currently have reimbursed access to ALYFTREK, and Vertex is working to secure access for eligible patients in additional countries. Vertex plans to share data from the global study of ALYFTREK in children 2 to 5 years of age and submit to global regulators in 2026. Vertex expects to initiate a pivotal study of ALYFTREK in children 1 to 2 years of age in 2026.
- **TRIKAFTA®:** Following positive results from the study of TRIKAFTA in patients one year to less than two years of age, reported in November 2025, Vertex expects to submit for approvals in this age group to global regulators, beginning in the first half of 2026.
- **Next-generation CFTR modulators:** Vertex has advanced VX-828, the first of the next-generation 3.0 CFTR corrector class, into a study in people with CF. Vertex expects to complete enrollment and dosing in this study and share data in the second half of 2026. Vertex also advanced VX-581, another corrector in this class, into a Phase 1 study in healthy volunteers.
- **VX-522:** Vertex is working to complete dosing in the multiple ascending dose (MAD) portion of the Phase 1/2 study of VX-522 and share data in the second half of 2026. VX-522 is a CFTR mRNA therapeutic that Vertex is developing in collaboration with Moderna for the approximately 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 CF in all target markets from approximately 109,000 to approximately 112,000, which includes an increase from 94,000 to approximately 97,000 people with CF in the core markets of U.S., Europe, Australia, and Canada.

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

- CASGEVY is approved in the U.S., the U.K., the EU, the Kingdom of Saudi Arabia, the Kingdom of Bahrain, Qatar, Canada, Switzerland, the United Arab Emirates, and Kuwait for patients 12 years and older with SCD or TDT.
- Vertex realized its goal for greater than $100 million of CASGEVY revenue in 2025, reflecting more than 60 patients receiving infusions of CASGEVY.
- At the American Society of Hematology (ASH) annual meeting in December 2025, Vertex presented positive data from the pivotal studies of CASGEVY in children ages 5 to 11 years old with SCD or TDT. Vertex expects to begin submitting in the first half of 2026 for approvals from global regulators. The U.S. Food and Drug Administration (FDA) awarded Vertex a Commissioner's National Priority Voucher for this pediatric submission, accelerating the timeline for review once the submission is complete.
- Taken together, Vertex expects these advances will result in significant CASGEVY revenue growth in 2026 and beyond.

Acute Pain – JOURNAVX®

- JOURNAVX (suzetrigine) is approved in the U.S. for the treatment of moderate-to-severe acute pain in adults.
- Since FDA approval on January 30, 2025, and pharmacy availability in March 2025, more than 500,000 JOURNAVX

prescriptions were written and filled in 2025 across both hospital and retail settings.
- Vertex secured commercial coverage for JOURNAVX with the remaining large national pharmacy benefit manager (PBM) and now has secured access for JOURNAVX with all three national PBMs. As of January 2026, over 200 million individuals now have access to JOURNAVX across commercial and government payers, representing two-thirds of U.S. covered lives – a significant achievement in the first year of product launch.
- Vertex plans to complete a regulatory submission in Canada for JOURNAVX for the treatment of moderate-to-severe acute pain in adults in the first half of 2026.
- With positive feedback on JOURNAVX's efficacy and tolerability and strong progress with payers, hospital, and physician adoption, Vertex expects the number of JOURNAVX prescriptions to more than triple in 2026 versus 2025.

Programs in Pivotal Development

Peripheral Neuropathic Pain (PNP)

- Vertex expects to complete enrollment in both Phase 3 studies of suzetrigine in diabetic peripheral neuropathy (DPN), a form of peripheral neuropathic pain (PNP), by the end of 2026.
- Vertex also continues to enroll and dose patients in a Phase 2 study of VX-993 in DPN.
- **Epidemiology and market opportunity update:** Vertex increased its estimates for the number of people with DPN in the U.S. from approximately 2 million to approximately 2.5 million, which reflects the aging U.S. population and increased prevalence of chronic pain in older age groups.

IgA Nephropathy (IgAN), Primary Membranous Nephropathy (pMN) and other B Cell-Mediated Diseases – povetacicept

- In the fourth quarter of 2025, Vertex initiated the rolling biologics license application (BLA) filing for U.S. accelerated approval of povetacicept in IgAN with submission of the first module. Vertex remains on track to complete the submission in the first half of 2026. Vertex is using a priority review voucher to expedite the review of the povetacicept BLA from ten months to six months, and the FDA has granted Breakthrough Therapy Designation for povetacicept in IgAN. The RAINIER Phase 3 study completed full enrollment in November.
- Vertex continues to enroll and dose patients in the Phase 2/3 OLYMPUS pivotal study of povetacicept in patients with pMN. The FDA has granted Fast Track designation for povetacicept in pMN, and the EMA has granted Priority Medicines (PRIME) designation.
- **Epidemiology and market opportunity update:** Vertex increased its estimates for the number of people with IgAN in the U.S. and Europe from approximately 300,000 to approximately 330,000 and estimates the global diagnosed population exceeds 1.5 million. For pMN, Vertex estimates the disease impacts approximately 150,000 people in the U.S. and Europe and more than 600,000 globally.

APOL1-Mediated Kidney Disease (AMKD) – inaxaplin

- In September, Vertex completed enrollment in the interim analysis cohort of the AMPLITUDE Phase 2/3 trial of inaxaplin in patients with primary AMKD and will conduct the pre-planned interim analysis once this cohort reaches 48 weeks of treatment. Vertex expects to share data from the interim analysis in late 2026 or early 2027. The AMPLITUDE study is on track to complete full enrollment in the second half of 2026.

Type 1 Diabetes (T1D)

- Vertex has completed enrollment in the Phase 1/2/3 study of zimislecel in people with T1D and has temporarily postponed completion of dosing in the study, pending an ongoing internal manufacturing analysis.
- Zimislecel has been granted Regenerative Medicine Advanced Therapy (RMAT) and Fast Track designations from the U.S. Food and Drug Administration, PRIME designation from the EMA, Breakthrough Medicine designation from the Kingdom of Saudi Arabia, and has secured an Innovation Passport under the Innovative Licensing and Access Pathway (ILAP) from the U.K. Medicines and Healthcare products Regulatory Agency (MHRA).

Programs in Mid-Stage Clinical Development

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

- Vertex is enrolling and dosing patients with ADPKD in the AGLOW Phase 2 proof- of-concept study. AGLOW is a 24-patient single-arm study that will evaluate the effect of VX-407 on height-adjusted total kidney volume (htTKV).

Myotonic Dystrophy Type 1 (DM1) – VX-670

- Vertex continues to enroll and dose the MAD portion of the GALILEO global Phase 1/2 clinical trial of VX-670 in people with DM1; the study is assessing both safety and efficacy. Vertex is on track to complete enrollment and dosing in the trial in mid-2026.

Generalized Myasthenia Gravis (gMG) – povetacicept

- Vertex expects to initiate a Phase 2 study of povetacicept for the treatment of generalized myasthenia gravis, another B cell-mediated disease, in the first half of 2026.

- **Epidemiology and market opportunity update:** Vertex estimates that the number of people with gMG is approximately 175,000 in the U.S. and Europe and more than 300,000 globally.

Additional Earlier Stage R&D Programs

- Consistent with its overall strategy, Vertex takes a portfolio approach to all of its programs, with additional assets or approaches in CF, SCD, TDT, pain, AMKD, T1D, DM1, and ADPKD in earlier stages of development. Additionally, Vertex is working on preclinical molecules with the potential to expand its leadership in existing disease areas, including assets targeting improved immunosuppression for zimislecel, gentler conditioning for CASGEVY, and inhibition of NaV1.7 in pain.

J.P. Morgan Healthcare Conference Presentation and Webcast
Dr. Kewalramani will present at the 44th Annual J.P. Morgan Healthcare Conference on Monday, January 12, 2026, at 5:15 p.m. ET/2:15 p.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 and conditions. The company has approved therapies for cystic fibrosis, sickle cell disease, transfusion-dependent beta thalassemia and acute pain, and it continues to advance clinical and research programs in these areas. 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 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 16 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 at www.vrtx.com or follow us on LinkedIn, Facebook, Instagram, YouTube and 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 expectations for the company’s CF program, including with respect to commercial expansion, expectations for advancement of the emerging renal franchise, plans to progress the company’s mid- and late-stage clinical pipeline, and beliefs that the company is well-positioned to serve more patients and deliver value for shareholders, expectations to expand access to ALYFTREK in additional countries, share data from the global study of ALYFTREK in patients 2 to 5 years of age and submit to global regulators in 2026, plans to initiate a pivotal study of ALYFTREK in patients 1 to 2 years of age in 2026, expectations to submit to global regulators for approval for TRIKAFTA in patients one year to less than two years of age beginning in the first half of 2026, expectations to complete enrollment and dosing in the VX-828 study of people with CF and share data in the second half of 2026, expectations with respect to Vertex’s next-generation, 3.0 CFTR corrector class, plans to complete dosing in the MAD portion of the Phase 1/2 study of VX-522 and share data from the study in the second half of 2026, expectations that VX-522 may treat the ~5,000 people with CF who cannot benefit from CFTR modulators, the company’s beliefs regarding CF epidemiology and market opportunities, expectations for CASGEVY, including with respect to beginning global regulatory submissions in the first half of 2026, beliefs regarding an accelerated timeline for review, and expectations for significant CASGEVY revenue growth in 2026 and beyond, expectations with respect to JOURNAVX, including with respect to tripling the number of JOURNAVX prescriptions in 2026 versus 2025, and plans to complete regulatory submissions in Canada for JOURNAVX in the first half of 2026, expectations regarding Vertex’s PNP program, including with respect to completing enrollment in both Phase 3 studies of suzetrigine in DPN by the end of 2026, plans with respect to the Phase 2 study of VX-993 in DPN, and the company’s beliefs regarding DPN epidemiology and market opportunities, expectations with respect to povetacicept and Vertex’s programs in IgAN, pMN, and other B cell-mediated diseases, including with respect completion of the BLA submission for povetacicept in IgAN in the U.S. in the first half of 2026, the anticipated expedited review period, plans to continue to enroll and dose the Phase 2/3 OLYMPUS pivotal study of povetacicept in pMN, expectations to initiate a Phase 2 study of povetacicept in gMG in the first half of 2026, and the company’s beliefs regarding epidemiology and market opportunities for IgAN, pMN, and gMG, expectations regarding inaxaplin and Vertex’s AMKD program, including with respect to conducting the pre-planned interim analysis once the cohort reaches 48 weeks of treatment, expectations to share data from the interim analysis in late 2026 or early 2027, and completing full enrollment in the AMPLITUDE study in the second half of 2026, expectations with respect to zimislecel and Vertex’s T1D program, including expectations regarding the temporary postponement of the Phase 1/2/3 study of zimislecel and plans for the ongoing internal manufacturing analysis, expectations regarding VX-407 and Vertex’s ADPKD program, and expectations regarding VX-670 and Vertex’s DM1 program, including with respect to completing enrollment and dosing in the GALILEO study in mid-2026, and the company’s beliefs with respect to additional assets or approaches in CF, SCD, TDT, pain, AMKD, T1D, DM1, and ADPKD, including working on preclinical molecules with the potential to expand Vertex’s leadership in existing disease areas, including assets targeting improved immunosuppression for zimislecel, gentler conditioning for CASGEVY, and inhibition of NaV1.7 in pain. 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 the company may be unable to successfully commercialize its marketed products, 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 data from the company's research and development programs may not support registration or further development of its potential medicines in a timely manner, or at all, due to safety, efficacy, or other reasons, that anticipated commercial launches may be delayed, if they occur at all, that external factors may have different or more significant impacts on the company's business or operations than the company currently expects, that regulatory submissions may not occur on the anticipated timeline, or at all, that discussions with regulators may cause delays in the company's 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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