Vertex and Republic of Ireland Expand Long-Term Cystic Fibrosis Medicines Reimbursement Agreement to Include Triple Combination Therapy for All Eligible Patients Ages 12 and Over Once Licensed

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LONDON--(BUSINESS WIRE)--Dec. 13, 2019-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today confirmed that, following recent collaborative discussions, it has negotiated an agreement with the Health Service Executive (HSE) in the Republic of Ireland to expand the existing long-term cystic fibrosis (CF) reimbursement agreement to include the triple combination regimen *(elexacaftor, tezacaftor and ivacaftor),* which is under review and pending approval by the European Medicines Agency, for all eligible patients ages 12 and older in line with the potential future licensed indication.

This expands the existing agreement which had already included access to current and future Vertex medicines for people in Ireland with CF of any age who:

- Have one of the specified mutations as included in the KALYDECO® marketing authorization (*G551D, G551S, S549R, G1244E, S1251N, G1349D, S1255P, G178R or S549N*) or
- Are homozygous for the *F508del* mutation or
- Are heterozygous for the *F508del* mutation and have a specified mutation (*P67L*, *D579G, D1152H, R117C, 711+3A→G, 2789+5G→A, L206W, S945L, 3272-26A→G, R352Q, S977F, 3849+10kbC→T, A455E or R1070W*) and people ages 18 and older and have an *R117H* mutation.

This innovative long-term agreement also enabled rapid access for people with these mutations if the labels of the existing medicines were expanded to cover additional age groups and if new Vertex medicines were approved for these populations.

Ludovic Fenaux, Senior Vice President, Vertex International, said, “With this expanded agreement, even more patients in the Republic of Ireland will be among the first in Europe to benefit from the triple combination treatment once the medicine is licensed.”

Triple combination treatment (*elexacaftor, tezacaftor and ivacaftor)* is not currently licensed for use in the European Union. Vertex submitted a Marketing Authorization Application to the European Medicines Agency on October 31, 2019.

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 four approved medicines in the U.S. and three approved medicines in Europe 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 pipeline of investigational small molecule medicines in other serious diseases where it has deep insight into causal human biology, including pain, alpha-1 antitrypsin deficiency, and APOL1-mediated kidney disease. In addition, Vertex has a rapidly expanding pipeline of genetic and cell therapies for diseases such as sickle cell disease, beta thalassemia, Duchenne muscular dystrophy and type 1 diabetes mellitus.

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, UK. 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 10 consecutive years on Science magazine's Top Employers list and top five on the 2019 Best Employers for Diversity list by Forbes.

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, the statements by Mr. Fenaux in the second paragraph of this press release and statements regarding the expansion of the existing long-term cystic fibrosis reimbursement agreement and Vertex’s Marketing Authorization Application submission to the European Medicines Agency. 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 development programs may not support registration or further development of its compounds due to safety,

efficacy or other reasons, obtaining approval and commercializing elexacaftor, tezacaftor and ivacaftor in Europe, developing additional medicines to treat cystic fibrosis, and other risks listed under Risk Factors in Vertex's annual report and subsequent quarterly reports filed with the Securities and Exchange Commission and available through the company's website at www.vrtx.com. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

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