Vertex Confirms Wales Offer Accepted for Access to All Licensed Cystic Fibrosis Medicines

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-Eligible patients in Wales will soon have access to ORKAMBI® (lumacaftor/ivacaftor) and SYMKEVI® (tezacaftor/ivacaftor), expanded access to KALYDECO® (ivacaftor) under same terms as NHS England agreement-

LONDON--(BUSINESS WIRE)--Nov. 13, 2019-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today confirms that NHS Wales has accepted an offer for all currently licensed Vertex cystic fibrosis (CF) medicines and any future indications of these medicines under the same terms as the recently announced agreement with NHS England.

This means that once the contract is finalized, patients with CF in Wales ages 2 years and older who have two copies of the *F508del* mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene can access ORKAMBI® (lumacaftor/ivacaftor) and CF patients ages 12 years and older who either have two copies of the *F508del* mutation or one copy of the *F508del* mutation and a copy of one of the other 14 licensed mutations can access SYMKEVI® (tezacaftor/ivacaftor) in combination with ivacaftor in the coming weeks.

The agreement also offers expanded access to KALYDECO® (ivacaftor) to include those patients ages 12 months and older who have one of the nine licensed gating mutations.

"Today's announcement is good news for the approximately 270 eligible cystic fibrosis patients in Wales who will soon have access to CFTR modulators to treat the underlying cause of their disease," said Ludovic Fenaux, Senior Vice President, Vertex International. "We thank the authorities in Wales for their collaboration in accepting this offer under the same terms as were recently announced in England."

About CF in the UK
Over 10,000 people in the UK have CF — the second highest number in the world. Over 430 people in Wales have CF. CF is a debilitating, life-shortening inherited condition that causes progressive damage to organs across the body from birth. Currently, there is no cure for CF and half of people in the UK with CF die before they are 32. The daily impact of treatment is significant. It can take up to four or more hours, involving nebulizers, physiotherapy and up to 70 tablets a day. CF accounts for 9,500 hospital admissions and over 100,000 hospital bed days a year. A third of these are used by children under 15.

About ORKAMBI® (lumacaftor/ivacaftor) and the *F508del* mutation
In people with two copies of the *F508del* mutation, the CFTR protein is not processed and trafficked normally within the cell, resulting in little-to-no CFTR protein at the cell surface. Patients with two copies of the *F508del* mutation are easily identified by a simple genetic test.

Lumacaftor/ivacaftor is a combination of lumacaftor, which is designed to increase the amount of mature protein at the cell surface by targeting the processing and trafficking defect of the F508del-CFTR protein, and ivacaftor, which is designed to enhance the function of the CFTR protein once it reaches the cell surface.

For complete product information, please see the Summary of Product Characteristics that can be found on www.ema.europa.eu.

About SYMKEVI® (tezacaftor/ivacaftor) in combination with ivacaftor
Some mutations result in CFTR protein that is not processed or folded normally within the cell, and that generally does not reach the cell surface. Tezacaftor is designed to address the trafficking and processing defect of the CFTR protein to enable it to reach the cell surface and ivacaftor is designed to enhance the function of the CFTR protein once it reaches the cell surface.

SYMKEVI is indicated for people with CF ages 12 and older who either have two copies of the *F508del* mutation or one copy of the *F508del* mutation and have one of the following 14 mutations in which the CFTR protein shows residual function: *P67L, R117C, L206W, R352Q, A455E, D579G, 711+3A→G, S945L, S977F, R1070W, D1152H, 2789+5G→A, 3272-26A→G,* or *3849+10kbC→T.*

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About KALYDECO® (ivacaftor)
KALYDECO® (ivacaftor) is the first medicine to treat the underlying cause of CF in people with specific mutations in the *CFTR*

gene. Known as a CFTR potentiator, ivacaftor is an oral medicine designed to keep CFTR proteins at the cell surface open longer to improve the transport of salt and water across the cell membrane, which helps hydrate and clear mucus from the airways.

KALYDECO is indicated in people ages 12 months and older who have one of the following mutations in the *CFTR* gene: *G551D, G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N* or *S549R*. KALYDECO is also indicated for the treatment of patients with CF ages 18 years and older who have an *R117H* mutation in the CFTR gene.

For complete product information, please see the Summary of Product Characteristics that can be found on www.ema.europa.eu.

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 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 medicines in other serious diseases where it has deep insight into causal human biology, such as sickle cell disease, beta thalassemia, pain, alpha-1 antitrypsin deficiency, Duchenne muscular dystrophy and APOL1-mediated kidney diseases.

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 fourth paragraph of this press release, statements regarding our expectations for the patient populations that will be able to access Vertex's medicines and the timing of such access, and statements about our expectations regarding a formal agreement in Northern Ireland. 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, 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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