Vertex Announces New Access Agreement with Scottish Government for ORKAMBI® (lumacaftor/ivacaftor) and SYMKEVI® (tezacaftor/ivacaftor)

September 12, 2019

Eligible patients in Scotland will immediately have access to ORKAMBI (lumacaftor/ivacaftor) and SYMKEVI (tezacaftor/ivacaftor) in combination with ivacaftor

LONDON--(BUSINESS WIRE)--Sep. 12, 2019-- Vertex Pharmaceuticals Incorporated (NASDAQ: VRTX) today announced that eligible cystic fibrosis patients living in Scotland will now have access to ORKAMBI® (lumacaftor/ivacaftor) and SYMKEVI® (tezacaftor/ivacaftor) in combination with ivacaftor following the signing of an access agreement.

“We would like to thank the Scottish authorities for their partnership and the collaborative and flexible way that we have worked together to find this access solution,” said Ludovic Fenaux, Senior Vice President, Vertex International. “It means that approximately 400 eligible cystic fibrosis patients in Scotland now have access to ORKAMBI or SYMKEVI.”

As part of the 5-year agreement Vertex has also committed to collecting real world data on these medicines that will support any future submissions to the Scottish Medicines Consortium (SMC).

Vertex cystic fibrosis medicines are reimbursed in 17 countries around the world including Austria, Australia, Denmark, Germany, the Republic of Ireland, Italy, the Netherlands, Sweden and the U.S.

About CF in Scotland
Approximately 900 people in Scotland live with CF. In the UK, the median age of death is 32 years. NHS Scotland estimates that one in 24 Scots have a CFTR mutation which, if carried by both parents, would lead to a child being born with CF.

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.

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 three 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'sInnovation 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 nine 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 in the third paragraph of the press release. 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 factors that could cause actual events or results to differ materially from those indicated 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 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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