French Authorities Approve National Reimbursement of ORKAMBI® (lumacaftor/ivacaftor) for Eligible People Ages Two and Older With Cystic Fibrosis

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LONDON--(BUSINESS WIRE)--Nov. 20, 2019-- Vertex Pharmaceuticals Incorporated (NASDAQ: VRTX) today announced that the French Authorities (Comité économique des produits de santé, or CEPS) have approved national reimbursement of ORKAMBI® (lumacaftor/ivacaftor) for people ages two and older with cystic fibrosis (CF) who have two copies of the *F508del* mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene.

ORKAMBI will be available for all eligible patients once the agreement has been published in the French Official Journal.

In France, ORKAMBI has been available to eligible patients ages 12 and older since December 2015 through a Temporary Use Authorization (Autorisations Temporaires d'Utilisation, or ATU) and a post-ATU interim access program. In addition to providing reimbursement for patients ages 12 and older, today's agreement also enables people ages 2 to 11 years to access ORKAMBI in France for the first time. All those who are currently prescribed ORKAMBI through the ATU will now receive it through this new agreement.

"With today's announcement, eligible patients across France can receive ORKAMBI through a national reimbursement agreement. Importantly, children with cystic fibrosis as young as two years of age with two copies of the *F508del* mutation will now benefit from this precision medicine," said Ludovic Fenaux, Senior Vice President, Vertex International. "We thank the French Authorities for their engagement and commitment that has resulted in an agreement to provide sustainable access to ORKAMBI for all eligible patients."

Vertex's CF medicines are reimbursed in 21 countries around the world, including recent agreements in Australia, England and Spain.

About Cystic Fibrosis
Cystic fibrosis (CF) is a rare, life-shortening genetic disease affecting approximately 75,000 people worldwide. CF is a progressive, multi-system disease that affects the lungs, liver, GI tract, sinuses, sweat glands, pancreas and reproductive tract. CF is caused by a defective and/or missing CFTR protein resulting from certain mutations in the *CFTR* gene. Children must inherit two defective *CFTR* genes — one from each parent — to have CF. While there are many different types of *CFTR* mutations that can cause the disease, the vast majority of all people with CF have at least one *F508del* mutation. These mutations, which can be determined by a genetic test, or genotyping test, lead to CF by creating non-working and/or too few CFTR proteins at the cell surface. The defective function and/or absence of CFTR protein results in poor flow of salt and water into and out of the cells in a number of organs. In the lungs, this leads to the buildup of abnormally thick, sticky mucus that can cause chronic lung infections and progressive lung damage in many patients that eventually leads to death. The median age of death is in the early 30s.

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 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 fourth paragraph of this press release and statements regarding our expectations for (i) the availability of ORKAMBI, and (ii) patients' eligibility for and access to ORKAMBI. 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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Source: Vertex Pharmaceuticals Incorporated

Vertex Pharmaceuticals Incorporated
Investors:
InvestorInfo@vrtx.com
or
617-961-7163

Media:
mediainfo@vrtx.com
or
International: +44 20 3204 5275
French: +33178421006
or
U.S.: 617-341-6992