September 25, 2015

Vertex Receives CHMP Positive Opinions for ORKAMBI™ (lumacaftor/ivacaftor) and KALYDECO® (ivacaftor) in the European Union

-CHMP recommends Marketing Authorization for lumacaftor in combination with ivacaftor for people with cystic fibrosis ages 12 and older with two copies of the F508del mutation-

-CHMP recommends Marketing Authorization for ivacaftor for children with cystic fibrosis ages 2 to 5 with 9 gating mutations & for people with cystic fibrosis ages 18 and older with the R117H mutation-

LONDON--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that the European Union Committee for Medicinal Products for Human Use (CHMP) issued a positive Opinion recommending Marketing Authorization of ORKAMBI™ (lumacaftor/ivacaftor), the first medicine to treat the underlying cause of cystic fibrosis (CF) in people ages 12 and older with two copies of the F508del mutation. In Europe, approximately 12,000 people with CF ages 12 and older have two copies of this mutation.

The CHMP also issued a positive Opinion recommending the indication of KALYDECO® (ivacaftor) be expanded to include children ages 2 to 5 with CF, in addition to the current approved use in people ages 6 and older, who have one of nine gating mutations in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene (*G551D, G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N* and *S549R*). A new weight-based oral granule formulation of ivacaftor (50 mg and 75 mg) that is mixed in soft foods or liquids was created for these younger children. The CHMP Opinion for the use of ivacaftor in children ages 2 to 5 included a recommendation to extend weight-based dosing of ivacaftor to children ages 6 to 11 who weigh less than 25 kg using the new oral granule formulation. In addition, the CHMP issued a positive Opinion recommending ivacaftor for use in people with CF ages 18 and older who have the R117H mutation. In Europe, approximately 125 children ages 2 to 5 have one of the nine gating mutations included in today's positive Opinion, and approximately 250 adults have the R117H mutation.

"Today's recommendations bring us closer to being able to help many more people with cystic fibrosis who currently do not have a medicine to treat the underlying cause of this chronic progressive disease," said Jeffrey Chodakewitz, M.D., Executive Vice President and Chief Medical Officer at Vertex. "However, we know that there are many more people with CF who are still waiting for treatment, and we remain committed to continuing to work toward our goal of creating new medicines for the vast majority of people with cystic fibrosis."

These positive CHMP Opinions are recommendations for approval to the European Commission, which then has the authority to approve medicines for the European Union. The European Commission generally follows the recommendations of the CHMP and typically issues Marketing Authorization within three months. If the CHMP's recommendations are approved, Vertex would then begin the country-by-country reimbursement approval process for each new medicine and indication.

About Cystic Fibrosis

Cystic fibrosis is a rare, life-threatening genetic disease affecting approximately 75,000 people in North America, Europe and Australia.

CF is caused by a defective or missing CFTR protein resulting from mutations in the *CFTR* gene. Children must inherit two defective *CFTR* genes — one from each parent — to have CF. There are approximately 2,000 known mutations in the *CFTR* gene. Some of these mutations, which can be determined by a genetic, or genotyping test, lead to CF by creating non-working or too few CFTR protein at the cell surface. The defective function or absence of CFTR proteins in people with CF results in poor flow of salt and water into and out of the cell 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. Today, the median predicted age of survival for a person with CF is between 34 and 47 years, but the median age of death remains in the mid-20s.

Collaborative History with Cystic Fibrosis Foundation Therapeutics, Inc. (CFFT)

Vertex initiated its CF research program in 1998 as part of a collaboration with CFFT, the nonprofit drug discovery and

development affiliate of the Cystic Fibrosis Foundation. Lumacaftor and ivacaftor were discovered by Vertex as part of this collaboration.

About Vertex

Vertex is a global biotechnology company that aims to discover, develop and commercialize innovative medicines so people with serious diseases can lead better lives. In addition to our clinical development programs focused on cystic fibrosis, Vertex has more than a dozen ongoing research programs aimed at other serious and life-threatening diseases.

Founded in 1989 in Cambridge, Mass., Vertex today has research and development sites and commercial offices in the United States, Europe, Canada and Australia. For five years in a row, Science magazine has named Vertex one of its Top Employers in the life sciences. For additional information and the latest updates from the company, please visit www.vrtx.com.

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, statements regarding the European Commission generally following the recommendations of the CHMP and typically issuing marketing approval within three months. While Vertex believes the forward-looking statements contained in this press release are accurate, 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 Vertex could experience unforeseen delays in obtaining marketing approval from the European Commission and the 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.

(VRTX-GEN)

View source version on businesswire.com: http://www.businesswire.com/news/home/20150925005249/en/

Vertex Pharmaceuticals Incorporated
Investors:
Michael Partridge, +1 617-341-6108
or
Kelly Lewis, +1 617-961-7530
or
Eric Rojas, +1 617-961-7205
or
Media:
mediainfo@vrtx.com
or
Europe & Australia: +44 20 3204 5275
or
North America: +1 617-341-6992

Source: Vertex Pharmaceuticals Incorporated