June 11, 2015

# Vertex Announces Data Presentations at European Cystic Fibrosis Society (ECFS) Conference

*-Analysis from TRAFFIC and TRANSPORT extension study of lumacaftor in combination with ivacaftor showed that improvements in lung function and other measures of disease were maintained through 48 weeks in people with cystic fibrosis who have two copies of the F508del mutation-*

BRUSSELS--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced data from PROGRESS, the long-term extension study of the investigational regimen ORKAMBI™ (lumacaftor/ivacaftor). The ongoing extension study enrolled people with cystic fibrosis (CF) ages 12 and older who have two copies of the F508del mutation and completed 24 weeks of treatment in the Phase 3 TRAFFIC and TRANSPORT studies (lumacaftor/ivacaftor combination and placebo treatment groups) and met certain other eligibility criteria. These data are included in one of nine presentations at the 38th European Cystic Fibrosis Society (ECFS) Conference, June 10-12, 2015, in Brussels, Belgium.

**Lumacaftor in combination with ivacaftor in patients with cystic fibrosis who are homozygous for the *F508del-CFTR* mutation** (ECFS Abstract WS01.3, oral presentation during *Workshop 1 - Strategies to correct CFTR defects*.)

One thousand thirty-one people who completed 24 weeks of treatment in either of the Phase 3 studies, TRAFFIC or TRANSPORT, entered the 96-week PROGRESS Phase 3 extension study in which everyone received one of two lumacaftor/ivacaftor combination regimens. An interim analysis was conducted once all patients completed 24 weeks in PROGRESS for a total of 48 total weeks of treatment (48 weeks of treatment with a combination regimen for patients who received a combination regimen in TRAFFIC and TRANSPORT; 24 weeks of treatment with a combination regimen for patients who received placebo in TRAFFIC and TRANSPORT).

These data showed that the initial improvements in lung function (percent predicted forced expiratory volume in one second, or ppFEV1) observed in the 24-week TRAFFIC and TRANSPORT studies among those treated with a lumacaftor/ivacaftor combination were sustained through 48 weeks of treatment across all patients. Reduced rates of pulmonary exacerbations and improvements in body mass index (BMI) and patient-reported respiratory symptoms as measured by the respiratory domain of the Cystic Fibrosis Questionnaire Revised (CFQ-R) were also maintained over 48 weeks. In addition, the pattern and magnitude of response observed after the initiation of combination treatment across all patients who received placebo in TRAFFIC and TRANSPORT and subsequently received a combination regimen in PROGRESS were similar to those seen among patients who received a combination regimen in TRAFFIC and TRANSPORT.

At the time of this analysis, the safety and tolerability results, including the type and frequency of adverse events and serious adverse events, were consistent with those observed in TRAFFIC and TRANSPORT, and no new safety concerns were identified. Over 48 weeks, the most common adverse events were infective pulmonary exacerbation, cough and increased sputum. The incidence of serious adverse events during PROGRESS was generally similar to TRAFFIC and TRANSPORT.

Other data presented at the Conference include:

- "Lumacaftor/ivacaftor combination therapy in CF patients homozygous for *F508del-CFTR* with severe lung dysfunction." Poster 143.
- "VX-661 in combination with ivacaftor in patients with cystic fibrosis and the *F508del-CFTR* mutation." ECFS Abstract WS01.4, oral presentation during *Workshop 1 - Strategies to correct CFTR defects*.
- "*R117H-CFTR* has a defect in channel gating activity that can be potentiated by ivacaftor." ECFS Abstract WS06.2, oral presentation during *Workshop 6 - Fixing ion transport*.
- "An open-label study of the safety, pharmacokinetics, and pharmacodynamics of ivacaftor in patients aged 2 to 5 years with cystic fibrosis and a *CFTR* gating mutation: the KIWI study." ECFS Abstract WS01.5, oral presentation during *Workshop 1 - Strategies to correct CFTR defects*.
- "Prevalence of cataracts in a population of cystic fibrosis patients homozygous for the *F508del* mutation." Poster 196.
- "Manifestation and progression of illness in young children with cystic fibrosis: a targeted literature review." Poster 186.

- "Frequency and costs of pulmonary exacerbations and association with % predicted $FEV_1$ in patients with cystic fibrosis." Poster 190.
- "Lung function and health care resource utilization in patients with cystic fibrosis." Poster 191.

**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 more than 1,900 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 proteins at the cell surface. The defective or missing CFTR protein results in poor flow of salt and water into and out of the cell in a number of organs, including the lungs. This leads to the build-up of abnormally thick, sticky mucus that can cause chronic lung infections and progressive lung damage 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. This collaboration was expanded to support the accelerated discovery and development of Vertex's CFTR modulators.

**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 data that will be presented at ECFS. 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 regulatory authorities may not approve, or approve on a timely basis, lumacaftor in combination with ivacaftor 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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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
Europe & Australia: +41 22 593 6066
or
North America: +1 617-341-6992

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