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Vertex Announces Presentation of Data at North American Cystic Fibrosis Conference

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that 10 abstracts from its cystic fibrosis (CF) research and development program will be presented at the 26th Annual North American Cystic Fibrosis Conference (NACFC) in Orlando, Fla., October 11 to 13, 2012. Previously announced data from a Phase 2 study of VX-809 combined with ivacaftor in people with the most common mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene, F508del, will be presented for the first time. Additional data from Vertex's work to discover and develop medicines that target the underlying cause of CF will also be presented, including data on KALYDECO™ (ivacaftor) in people with CF who have the G551D mutation.

The accepted abstracts are now available on the NACFC website at: https://www.nacfconference.org/.

Vertex Abstracts *(Oral presentations will also be presented as posters)*

1. "Hyperpolarized Gas MRI of Ivacaftor Therapy in Persons with Cystic Fibrosis and the *G551D-CFTR* Mutation." Poster #196. An oral presentation is scheduled for October 11, 2012, 10:55 a.m. EDT.

2. "The Investigational CFTR Corrector, VX-809 (Lumacaftor) Co-Administered with the Oral Potentiator Ivacaftor Improved CFTR and Lung Function in *F508DEL* Homozygous Patients: Phase II Study Results." Poster #260. An oral presentation is scheduled for October 11, 2012, 11:40 a.m. EDT.

3. "Identification and Characterization of CFTR Corrector VRT-534 (C-18)." Poster #30.

4. "Ivacaftor Potentiates Multiple Mutant Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) Forms." Poster #31.

5. "Long-Term Safety and Efficacy of Ivacaftor in Persons with Cystic Fibrosis who have the *G551D-CFTR* Mutation." Poster #211.

6. "Patient-Reported Outcomes in Phase 3 Trials of Ivacaftor in Subjects with CF who have the *G551D-CFTR* Mutation." Poster #212.

7. "Nutritional Status Measures Among Persons with CF Carrying the *G551D-CFTR* Mutation who Received Ivacaftor or Placebo in Phase 3 Clinical Trials." Poster #214.

8. "Lung Clearance Index to Evaluate the Effect of Ivacaftor on Lung Function in Subjects with CF who have the *G551D-CFTR* Mutation and Mild Lung Disease." Poster #249.

9. "Exposure-Response Relationship for FEV_1 and Sweat Chloride in Patients with Cystic Fibrosis Treated with Ivacaftor, a CFTR Potentiator." Poster #235.

10. "Clinical Pharmacology Profile of Ivacaftor, a CFTR Potentiator." Poster #236.

About KALYDECO

KALYDECO™ (ivacaftor) is the first treatment to target the underlying cause of CF in people with the G551D mutation in the *CFTR* gene. Known as a CFTR potentiator, KALYDECO is an oral medicine that aims to help the CFTR protein function more normally once it reaches the cell surface, to help hydrate and clear mucus from the airways. KALYDECO (150mg, q12h) was first approved by the U.S. Food and Drug Administration in January 2012 and by the European Medicines Agency in July 2012, for use in people with CF ages 6 and older who have at least one copy of the G551D mutation in the *CFTR* gene.

Vertex retains worldwide rights to develop and commercialize KALYDECO. KALYDECO is under Priority Review by the Therapeutic Product Directorate (TPD) of Health Canada, and an application for review has been submitted to the Therapeutic Goods Administration (TGA) of Australia.

Indication and Important Safety Information for KALYDECO (ivacaftor)

KALYDECO (150mg tablets) is indicated for the treatment of cystic fibrosis (CF) in patients age 6 years and older who have a G551D mutation in the CFTR gene.

KALYDECO is not for use in people with CF due to other mutations in the CFTR gene. It is not effective in CF patients with two copies of the F508del mutation (F508del/F508del) in the CFTR gene.

High liver enzymes (transaminases, ALT and AST) have been reported in patients receiving KALYDECO. It is recommended that ALT and AST be assessed prior to initiating KALYDECO, every 3 months during the first year of treatment, and annually thereafter. Patients who develop increased transaminase levels should be closely monitored until the abnormalities resolve. Dosing should be interrupted in patients with ALT or AST of greater than 5 times the upper limit of normal. Following resolution of transaminase elevations, consider the benefits and risks of resuming KALYDECO dosing. Moderate transaminase elevations are common in subjects with CF. Overall, the incidence and clinical features of transaminase elevations in clinical trials was similar between subjects in the KALYDECO and placebo treatment groups. In the subset of patients with a medical history of elevated transaminases, increased ALT or AST have been reported more frequently in patients receiving KALYDECO compared to placebo.

Use of KALYDECO with medicines that are strong CYP3A inducers such as the antibiotics rifampin and rifabutin; seizure medications (phenobarbital, carbamazepine, or phenytoin); and the herbal supplement St. John's Wort substantially decreases exposure of KALYDECO which may diminish effectiveness. Therefore, co-administration is not recommended.

The dose of KALYDECO must be adjusted when concomitantly used with potent and moderate CYP3A inhibitors.

KALYDECO can cause serious adverse reactions including abdominal pain and high liver enzymes in the blood. The most common side effects associated with KALYDECO include headache; upper respiratory tract infection (the common cold), including sore throat, nasal or sinus congestion, and runny nose; stomach (abdominal) pain; diarrhea; rash; and dizziness. These are not all the possible side effects of KALYDECO. A list of the adverse reactions can be found in the full product labeling for each country where KALYDECO is approved. Patients should tell their healthcare providers about any side effect that bothers them or doesn't go away.

Please see full U.S. Prescribing Information for KALYDECO at www.KALYDECO.com and the EU Summary of Product Characteristics for KALYDECO at http://goo.gl/N3Tz4.

About Cystic Fibrosis

Cystic fibrosis is a rare, life-threatening genetic disease affecting approximately 30,000 people in the United States and 70,000 people worldwide. Today, the median predicted age of survival for a person with CF is approximately 38 years in the United States, but the median age of death remains in the mid-20s.

CF is caused by defective or missing CFTR proteins 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 1,800 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 absence of working CFTR proteins results in poor flow of salt and water into and out of the cell in a number of organs, including the lungs.

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 the Cystic Fibrosis Foundation

The Cystic Fibrosis Foundation is the world's leader in the search for a cure for cystic fibrosis. The Foundation funds more CF research than any other organization and nearly every CF drug available today was made possible because of Foundation support. Based in Bethesda, Md., the Foundation also supports and accredits a national care center network that has been recognized by the National Institutes of Health as a model of care for a chronic disease. The CF Foundation is a donor-supported nonprofit organization. For more information, visit www.cff.org.

About Vertex

Vertex creates new possibilities in medicine. Our team discovers, develops and commercializes innovative therapies so people with serious diseases can lead better lives.

Vertex scientists and our collaborators are working on new medicines to cure or significantly advance the treatment of hepatitis C, cystic fibrosis, rheumatoid arthritis and other life-threatening diseases.

Founded more than 20 years ago in Cambridge, Mass., we now have ongoing worldwide research programs and sites in the U.S., U.K. and Canada. Today, Vertex has more than 2,000 employees around the world, and for three years in a row, *Science* magazine has named Vertex one of its Top Employers in the life sciences.

VRTX — GEN

Vertex Pharmaceuticals Incorporated
Media:
Dawn Kalmar
Megan Goulart
617-444-6992
mediainfo@vrtx.com
or
Investors:
Michael Partridge, 617-444-6108
or
Kelly Lewis, 617-444-7530

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