



January 31, 2012

ADDING MULTIMEDIA FDA Approves KALYDECO™ (ivacaftor), the First Medicine to Treat the Underlying Cause of Cystic Fibrosis

- FDA approval received 3 months after submission of New Drug Application -
- KALYDECO approved to treat people with CF who have a specific genetic mutation -
- Vertex launches a comprehensive financial assistance and patient support program -

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) announced today that the U.S. Food and Drug Administration (FDA) has approved KALYDECO™ (ivacaftor), the first medicine to treat the underlying cause of cystic fibrosis (CF), a rare, genetic disease. KALYDECO (kuh-LYE-deh-koh) is approved for people with CF ages 6 and older who have at least one copy of the G551D mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene. Approximately 1,200 people in the United States, or 4 percent of those with CF, are believed to have this mutation. KALYDECO was granted approval in approximately three months, making it one of the fastest FDA approvals ever and marking the second approval of a new medicine from Vertex in less than a year. The company has established a financial assistance and patient support program to help get KALYDECO to eligible patients for whom it is prescribed. KALYDECO was discovered as part of a collaboration with Cystic Fibrosis Foundation Therapeutics, Inc., the nonprofit drug discovery and development affiliate of the Cystic Fibrosis Foundation.



KALYDECO carton, bottle, and pills (Photo: Business Wire)
more people with cystic fibrosis."

Vertex is ready to support the introduction of KALYDECO and will begin shipping it to pharmacies in the United States this week. The company will host a conference call for investors and media today, January 31, 2012, at 12:15 p.m. ET to provide more information on KALYDECO availability, price and the financial assistance and patient support program.

"More than 13 years ago we set out to change the lives of people with cystic fibrosis by developing new medicines that address the underlying cause of this rare and devastating disease," said Jeffrey Leiden, M.D., Ph.D., Vertex's incoming President and Chief Executive Officer. "KALYDECO represents a major advance in the treatment of cystic fibrosis for people with a specific type of this disease. But our work isn't done. With the ongoing support of doctors, patients and the Cystic Fibrosis Foundation, we're making progress toward our ultimate goal of developing additional medicines to help many

The approval of KALYDECO was based on data from two Phase 3 studies of people with CF who have at least one copy of the G551D mutation. Those who were treated with KALYDECO experienced significant and sustained improvements in lung function as well as other disease measures, including weight gain and certain quality of life measurements, compared to those who received placebo. People who took KALYDECO also experienced significantly fewer pulmonary exacerbations, which are periods of worsening in the signs and symptoms of the disease that often require treatment with antibiotics and hospital visits. Fewer people in the KALYDECO treatment groups discontinued treatment due to adverse events than in the placebo groups. The majority of adverse events associated with KALYDECO were mild to moderate. Adverse events commonly observed in those taking KALYDECO included headache, upper respiratory tract infection (common cold), stomach pain and diarrhea.

"Advances in cystic fibrosis treatment have helped manage symptoms of the disease, however people with cystic fibrosis still have a hard time staying healthy and being active," said Bonnie Ramsey, M.D., Director of the Center for Clinical and Translational Research at Seattle Children's Research Institute and principal investigator for one of the Phase 3 KALYDECO trials. "KALYDECO is a fundamental shift in the way cystic fibrosis is treated. In people with a specific genetic mutation, KALYDECO helped them breathe more easily, gain weight and generally feel better."

"Together, we're changing the lives of people with cystic fibrosis," said Robert J. Beall, Ph.D., President and CEO of the Cystic Fibrosis Foundation. "We now have a medicine that treats the underlying cause of the disease in people with the G551D mutation. KALYDECO also provides us with a roadmap for exploring additional targeted approaches to treatment for all people with cystic fibrosis."

Cystic fibrosis is a rare, life-threatening genetic disease for which there is no cure. CF is caused by defective or missing CFTR proteins resulting from mutations in the *CFTR* gene. CFTR proteins act as channels at the cell surface that control the flow of salt and water across the cells. When the defective CFTR protein does not work properly at the cell surface, abnormally thick, sticky mucus builds up in the lungs. The digestive tract and a number of other organs are also affected. KALYDECO, an oral medicine known as a CFTR potentiator, helps the CFTR protein function more normally once it reaches the cell surface. KALYDECO targets the abnormal CFTR protein channels and opens them to allow chloride ions to move into and out of the cell, which helps thin the mucus so it can hydrate and protect the airways, and keeps them from getting clogged and then infected.

Because KALYDECO targets a specific genetic mutation, a person's genotype should be known before this new medicine is prescribed. Genetic testing is widely available and FDA-cleared tests are available for people with CF whose genotype is unknown. According to the 2010 Cystic Fibrosis Foundation's Patient Registry, nearly 92 percent of people with CF have already had their CF mutations identified.

KALYDECO by itself works in a subset of people with CF, but research is ongoing to explore a similar targeted approach using a combination of medicines, including KALYDECO, to treat the most common form of the disease.

Helping People with CF Get KALYDECO

The people who work at Vertex understand that medicines can only help patients who can get them. To that end, the company offers a comprehensive financial assistance and patient support program. A specially-trained and dedicated Vertex team will provide one-on-one support to help eligible patients who are prescribed KALYDECO understand their insurance benefits and the resources that are available to help them.

For eligible patients, the program also includes the following:

- **Free Medicine Program:** Vertex will provide KALYDECO for free to people who do not have insurance and have an annual household income of \$150,000 or less; and
- **Co-Pay Assistance Program:** For patients with commercial insurance plans that cover KALYDECO and who are enrolled in the Guidance and Patient Support, or GPS, program, there will be a minimal out-of-pocket obligation after which Vertex will help cover co-pay or co-insurance costs up to 30 percent of the list price of the medicine. There is no income limit to be eligible for this program.

Some patients are not eligible for company co-pay support because they have Medicare or Medicaid coverage or live in Massachusetts. There are independent non-profit copay assistance foundations that may be able to help those patients with their out-of-pocket costs.

More information about this program is available by calling 1-877-7-KALYDECO (877-752-5933) or visiting www.VertexGPS.com.

About KALYDECO

KALYDECO is the first treatment to target the underlying cause of CF. The Phase 3 studies evaluated KALYDECO in people with CF ages 6 and older who had at least one copy of the G551D mutation. PERSIST, a Phase 3, open-label, 96-week extension study, is underway to evaluate the long-term safety and durability of treatment with KALYDECO. This ongoing study enrolled people who completed 48 weeks of treatment in either Phase 3 study (placebo and KALYDECO treatment groups) and met other eligibility criteria. KALYDECO will be taken as one 150-mg tablet twice daily (every 12 hours).

Vertex retains worldwide rights to develop and commercialize KALYDECO. In October 2011, Vertex submitted a marketing authorization application to the European Medicines Agency (EMA) for KALYDECO and has received agreement from the EMA for accelerated assessment in Europe. The EMA regulatory review is ongoing.

Indication and Important Safety Information

KALYDECO is a prescription medicine used for the treatment of cystic fibrosis (CF) in patients ages 6 years and older who have a certain mutation in their CF gene called the G551D mutation.

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

It is not known if KALYDECO is safe and effective in children under 6 years of age.

KALYDECO should not be used with certain medicines, including the antibiotics rifampin and rifabutin; seizure medications (phenobarbital, carbamazepine, or phenytoin); and the herbal supplement St. John's Wort.

KALYDECO can cause serious side effects. High liver enzymes in the blood have occurred in patients taking KALYDECO. Regular assessment is recommended.

The most common side effects associated with KALYDECO include headache; upper respiratory tract infection (common cold) including sore throat, nasal or sinus congestion, and runny nose; stomach (abdominal) pain; diarrhea; rash; nausea; and dizziness.

These are not all the possible side effects of KALYDECO. Patients should tell their healthcare providers about any side effect that bothers them or doesn't go away.

Please see full Prescribing Information for KALYDECO at www.KALYDECO.com.

Conference Call for Media and Investors

Vertex will host a conference call and webcast today, January 31, 2012 at 12:15 p.m. ET to provide more information about today's approval, the price of KALYDECO and Vertex's new financial assistance and patient support program. The conference call will be webcast live and a link to the webcast may be accessed from the 'Events & Presentations' page of Vertex's website at www.vrtx.com.

To listen to the live call on the telephone, dial 1-877-250-8889 (United States and Canada) or 1-720-545-0001 (International). To ensure a timely connection, it is recommended that users register at least 15 minutes prior to the scheduled webcast.

The conference ID number for the live call and replay is 48426093.

The call will be available for replay via telephone commencing January 31, 2012 at 3:00 p.m. ET running through 5:00 p.m. ET on February 7, 2012. The replay phone number for the United States and Canada is 1-855-859-2056. The international replay number is 1-404-537-3406.

Following the live webcast, an archived version will be available on Vertex's website until 5:00 p.m. ET on February 14, 2012. Vertex is also providing a podcast MP3 file available for download on the Vertex website at www.vrtx.com.

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 but the median age of death remains in the mid-20s. 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 across cell membranes in a number of organs, including the lungs. This leads to the buildup of abnormally thick, sticky mucus that can cause chronic lung infections and progressive lung damage.

In some people, CFTR proteins are present at the cell surface but do not work properly. One type of this dysfunction is known as the G551D mutation. Approximately 4 percent of those with CF, or about 1,200 people in the United States, are believed to have this mutation. An estimated 1,000 people in Europe have the G551D mutation.

In people with the most common mutation in the *CFTR* gene, F508del, the CFTR protein does not reach the cell surface in normal amounts and the CFTR proteins that reach the surface do not work correctly. Nearly 90 percent of people with CF have at least one copy of the F508del mutation; approximately half of those with CF have two copies. KALYDECO is not effective in CF patients who have two copies of the F508del mutation in the *CFTR* gene.

Vertex's Ongoing CF Research and Development Program

KALYDECO has been approved by the FDA for people with CF ages 6 and older who have at least one copy of the G551D mutation. Vertex is planning to begin additional studies this year to evaluate KALYDECO in children with CF as young as 2 years old and in people with CF who have the R117H mutation or gating mutations that were not evaluated in the previous Phase 3 studies.

Enrollment is ongoing in the second part of a Phase 2 clinical trial of combination regimens of KALYDECO and VX-809, a CFTR corrector, in people with the most common mutation in CF, known as F508del. In addition, the company plans to begin Phase 2 development of VX-661, a second CFTR corrector, in the first quarter of 2012.

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, epilepsy and other life-threatening diseases.

Founded more than 20 years ago in Cambridge, MA, 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 *Science* magazine named Vertex number one on its 2011 list of Top Employers in the life sciences.

Special Note Regarding Forward-Looking Statements

This press release contains forward-looking statements, as defined in the Private Securities Litigation Reform Act of 1995, as amended, including statements regarding (i) Vertex being ready to support the introduction of KALYDECO and beginning to ship it to pharmacies this week; (ii) Vertex's financial assistance and patient support programs; (iii) the progress Vertex is making toward its ultimate goal of developing additional medicines to help many more people with cystic fibrosis; (iv) the roadmap provided by KALYDECO for exploring additional targeted approaches to treatment for all people with cystic fibrosis; (v) the ongoing research to explore a targeted approach using a combination of medicines, including KALYDECO, to treat the most common form of the disease and (vi) planned additional clinical trials of KALYDECO in children as young as 2 years old and people with CF who have the R117H mutation and gating mutations that were not evaluated in previous Phase 3 clinical trials. While the company 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, risks related to the commercialization of KALYDECO and development of additional medicines to treat cystic fibrosis 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 Vertex'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)

Photos/Multimedia Gallery Available: <http://www.businesswire.com/cgi-bin/mmg.cgi?eid=50150557&lang=en>

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