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Vertex Announces a Signed Letter of Intent to Enable Public Reimbursement of KALYDECO® (ivacaftor) in Canada for Eligible People with Cystic Fibrosis

-Before patients can get access through public reimbursement, each participating province or territory must decide to reimburse KALYDECO through its individual drug program-

-Approximately 100 people ages 6 and older in Canada have the G551D mutation -

BOSTON--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) announced today that it has signed a letter of intent with the pan-Canadian Pricing Alliance (pCPA) to enable the public reimbursement of KALYDECO® (ivacaftor) for the treatment of eligible Canadians with cystic fibrosis (CF) ages 6 and older who have the G551D mutation. The letter of intent represents an agreement in principle with the pCPA regarding the public reimbursement of KALYDECO in Canada. However, before patients can get access through public reimbursement, each participating province or territory must decide to reimburse KALYDECO through its individual drug program. In Canada, there are approximately 100 people ages 6 and older with this specific mutation. KALYDECO is the first medicine to treat the underlying cause of cystic fibrosis for people with the G551D mutation in the *CFTR* gene.

Cystic fibrosis is a rare genetic disease for which there is no cure. CF is caused by defective or missing CFTR proteins that result from mutations in the *CFTR* gene. 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, including the lungs. KALYDECO facilitates increased chloride transport by potentiating the channel-open probability (or gating) of the CFTR protein.

"The letter of intent signed today with the pan-Canadian Pricing Alliance is an important step toward eligible Canadians receiving KALYDECO through public reimbursement. However, our work is not complete until each province has added KALYDECO to its individual drug program to ensure people can get access to this medicine," said Stuart Arbuckle, Executive Vice President and Chief Commercial Officer for Vertex. "We share the urgency of the CF community to bring this process to a successful conclusion, and we will work as quickly as the provinces are able to so that people can receive KALYDECO without delay."

The process to add KALYDECO to drug programs at the provincial and territorial level is ongoing. The letter of intent does not include the province of Quebec, which does not participate in the pCPA process.

KALYDECO is now available to eligible people with CF in more than 15 countries around the world, including the United States, England, Scotland, Northern Ireland, Wales, the Republic of Ireland, France, Germany, the Netherlands, Austria, Denmark, Sweden, Norway, Greece, Italy and Switzerland. KALYDECO was approved in Australia in July 2013, however public reimbursement discussions are ongoing.

About KALYDECO (ivacaftor)

KALYDECO (ivacaftor) is the first medicine to treat the underlying cause of CF in people with specific mutations 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 for use in people with CF ages 6 and older who have at least one copy of the G551D mutation and in February 2014 for use in people with CF ages 6 and older who have the following additional CFTR mutations: G178R, S549N, S549R, G551S, G1244E, S1251N, S1255P and G1349D. In Canada, KALYDECO was first approved in November 2012 for use in people with CF ages 6 and older who have at least one copy of the G551D mutation and in June 2014 for use in people with CF ages 6 and older who have the following additional CFTR mutations: G178R, S549N, S549R, G551S, G1244E, S1251N, S1255P, G1349D and G970R.

KALYDECO was approved by the European Medicines Agency in July 2012 and by the Therapeutic Goods Administration in Australia in July 2013 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.

About Cystic Fibrosis

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

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 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, including the lungs. This leads to the buildup of abnormally thick, sticky mucus that can cause chronic lung infections and progressive lung damage.

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 four 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.

Vertex's press releases are available at www.vrtx.com.

About Vertex in Canada

In 2009, Vertex established a research and development site in Laval, Quebec through the acquisition of Virochem Pharma Inc. Vertex employs approximately 75 people across Canada and has established Commercial and Medical teams in Canada to support the use of KALYDECO.

INDICATION AND IMPORTANT SAFETY INFORMATION FOR KALYDECO™ (ivacaftor)

Ivacaftor (150 mg 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.

In the United States, ivacaftor is also indicated for the treatment of CF in patients age 6 and older who have one of the following mutations in the *CFTR* gene: *G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N, or S549R*. In Canada, ivacaftor is indicated for these same mutations and additionally for *G970R*.

Ivacaftor is not effective in patients with CF with 2 copies of the *F508del* mutation (*F508del/F508del*) in the *CFTR* gene. The safety and efficacy of ivacaftor in children with CF younger than 6 years of age have not been established.

Elevated liver enzymes (transaminases; ALT and AST) have been reported in patients receiving ivacaftor. It is recommended that ALT and AST be assessed prior to initiating ivacaftor, 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 ivacaftor dosing.

Use of ivacaftor 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 ivacaftor and may diminish effectiveness. Therefore, co-administration is not recommended.

The dose of ivacaftor must be adjusted when used concomitantly with strong and moderate CYP3A inhibitors or when used in patients with moderate or severe hepatic disease.

Ivacaftor can cause serious adverse reactions including abdominal pain and high liver enzymes in the blood. The most common side effects associated with ivacaftor 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 ivacaftor. A list of the adverse reactions can be found in the product labeling for each country where ivacaftor is approved. Patients should tell their healthcare providers about any side effect that bothers them or does not go away.

Please see KALYDECO U.S. Prescribing Information, EU Summary of Product Characteristics, Canadian Product Monograph, Australian Consumer Medicine Information and Product Information, Swiss Prescribing Information and Patient Information, and the New Zealand Datasheet and Consumer Medicine Information.

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, Mr. Arbuckle's statements in the third paragraph of the press release, and the information provided regarding the reimbursement approval processes for each participating province and territory in Canada. 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 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, the 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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