



March 6, 2017

Vertex to Acquire CTP-656 from Concert Pharmaceuticals for the Treatment of Cystic Fibrosis

-Vertex to develop CTP-656 for potential use in future combination regimens aimed at treating the underlying cause of CF-

-Concert to receive \$160 million in cash with potential for \$90 million in future regulatory approval milestone payments-

BOSTON--(BUSINESS WIRE)-- [Vertex Pharmaceuticals Incorporated](#) (Nasdaq: VRTX) today announced that it has signed a definitive asset purchase agreement to acquire CTP-656 from Concert Pharmaceuticals (Nasdaq: CNCE). CTP-656 is an investigational cystic fibrosis transmembrane conductance regulator (CFTR) potentiator that has the potential to be used as part of future once-daily combination regimens of CFTR modulators that treat the underlying cause of cystic fibrosis (CF). As part of the agreement, Vertex will pay Concert \$160 million in cash for all worldwide development and commercialization rights to CTP-656. If CTP-656 is approved as part of a combination regimen to treat CF, Concert could receive up to an additional \$90 million in milestones based on regulatory approval in the U.S. and reimbursement in the UK, Germany or France. The agreement is subject to approval by Concert's shareholders and the expiration of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act. Concert's Board of Directors unanimously supports the transaction and recommends that Concert's shareholders vote in favor of it.

"Our vision is to develop the most effective and convenient medicines for people with CF," said Jeffrey Chodakewitz, M.D., Executive Vice President and Chief Medical Officer at Vertex. "We look forward to exploring once-daily regimens that combine CTP-656 with other potential medicines from our broad CF pipeline that treat the underlying cause of the disease."

CTP-656 was developed by Concert through the application of deuterium chemistry to modify Vertex's CFTR potentiator, ivacaftor. Ivacaftor was discovered by Vertex scientists and is approved in the U.S., Europe, Canada and Australia for people with CF who have specific mutations in the *CFTR* gene. CTP-656 has the potential to play a key role in future once-daily combination regimens to treat CF. Concert is currently conducting a Phase 2 study of CTP-656 in people with CF who have gating mutations. As part of the agreement, Vertex will acquire rights to all of Concert's other CF research and preclinical programs.

INDICATION AND IMPORTANT SAFETY INFORMATION FOR KALYDECO® (ivacaftor)

KALYDECO (ivacaftor) is a prescription medicine used for the treatment of cystic fibrosis (CF) in patients age 2 years and older who have one of the following mutations in their CF gene: *G551D*, *G1244E*, *G1349D*, *G178R*, *G551S*, *S1251N*, *S1255P*, *S549N*, *S549R*, or *R117H*. KALYDECO is not for use in people with CF due to other mutations in the CF gene. KALYDECO is not effective in patients with CF 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 2 years of age.

Patients should not take KALYDECO if they are taking certain medicines or herbal supplements such as: the antibiotics rifampin or rifabutin; seizure medications such as phenobarbital, carbamazepine, or phenytoin; or St. John's wort.

Before taking KALYDECO, patients should tell their doctor if they: have liver or kidney problems; drink grapefruit juice, or eat grapefruit or Seville oranges; are pregnant or plan to become pregnant because it is not known if KALYDECO will harm an unborn baby; and are breastfeeding or planning to breastfeed because it is not known if KALYDECO passes into breast milk.

KALYDECO may affect the way other medicines work, and other medicines may affect how KALYDECO works. Therefore the dose of KALYDECO may need to be adjusted when taken with certain medications. Patients should especially tell their doctor if they take antifungal medications such as ketoconazole, itraconazole, posaconazole, voriconazole, or fluconazole; or antibiotics such as telithromycin, clarithromycin, or erythromycin.

KALYDECO can cause dizziness in some people who take it. Patients should not drive a car, use machinery, or do anything that needs them to be alert until they know how KALYDECO affects them. Patients should avoid food containing grapefruit or Seville oranges while taking KALYDECO.

KALYDECO can cause serious side effects including:

High liver enzymes in the blood have been reported in patients receiving KALYDECO. The patient's doctor will do blood tests to check their liver before starting KALYDECO, every 3 months during the first year of taking KALYDECO, and every year while taking KALYDECO. For patients who have had high liver enzymes in the past, the doctor may do blood tests to check the liver more often. Patients should call their doctor right away if they have any of the following symptoms of liver problems: pain or discomfort in the upper right stomach (abdominal) area; yellowing of their skin or the white part of their eyes; loss of appetite; nausea or vomiting; or dark, amber-colored urine.

Abnormality of the eye lens (cataract) has been noted in some children and adolescents receiving KALYDECO. The patient's doctor should perform eye examinations prior to and during treatment with KALYDECO to look for cataracts. The most common side effects include headache; upper respiratory tract infection (common cold), which includes 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. **Please click [here](#) to see the full Prescribing Information for KALYDECO (ivacaftor).**

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 test, lead to CF by creating defective or too few CFTR proteins at the cell surface. The defective or missing CFTR protein results in poor flow of salt and water into or 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 in many patients that eventually leads to death. The median predicted age of survival for a person born today with CF is 41 years, but the median age of death is 27 years.

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 seven 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, Dr. Chodakewitz's statements in the second paragraph of the press release and statements regarding (i) approval by Concert's shareholders and the expiration of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act, and (ii) potential milestone payments. 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 to differ materially from those indicated by such forward-looking statements. These 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.

Additional Information about the Transaction and Where to Find It

This press release is being made in respect of the proposed asset purchase with Concert. The proposed asset purchase and the asset purchase agreement will be submitted to Concert's shareholders for their consideration and approval. In connection with the proposed asset purchase, Concert will file a proxy statement with the SEC. This press release does not constitute a solicitation of any vote or proxy from any shareholder of Concert. Investors are urged to read the proxy statement carefully and in its entirety when it becomes available and any other relevant documents or materials filed or to be filed with the SEC or incorporated by reference in the proxy statement, because they will contain important information

about the proposed asset sale. The definitive proxy statement will be mailed to Concert's shareholders. In addition, the proxy statement and other documents will be available free of charge at the SEC's internet website, www.sec.gov. When available, the proxy statement and other pertinent documents may also be obtained free of charge at the [Investors](http://www.concertpharma.com) section of Concert's website, www.concertpharma.com, or by directing a written request to Concert Pharmaceuticals, Inc., Attn: Investor Relations, in writing, at 99 Hayden Ave, #500, Lexington, MA 02421.

Certain Information Concerning Participants

Vertex and its directors, executive officers and other members of management and employees may be deemed to be participants in the solicitation of proxies in connection with the proposed asset purchase. Information about Vertex's directors and executive officers is included in Vertex's Annual Report on Form 10-K for the year ended December 31, 2016 filed with the SEC on February 23, 2017.

(VRTX-GEN)

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