Vertex Announces Access Contract in Denmark for Current and Future Cystic Fibrosis Medicines

October 1, 2018

- First-of-its-kind contract with the Danish pharmaceutical and procurement organization, Amgros, is effective from today -

- Vertex also announces reimbursement in Austria for ORKAMBI® (lumacaftor/ivacaftor) to treat patients ages 6 through 11 with two copies of the F508del mutation -

LONDON--(BUSINESS WIRE)--Oct. 1, 2018-- Vertex Pharmaceuticals (Europe) Limited today announced it has entered into an innovative access contract with the Danish pharmaceutical and procurement organization, Amgros. Effective from today, this first-of-its-kind contract includes current and future CFTR modulator medicines, and means eligible Danish cystic fibrosis (CF) patients will have access to all current medicines as well as future medicines after regulatory approval.

“We’re delighted to have collaborated with Amgros to finalize this pioneering contract,” said Ludovic Fenaux, Senior Vice President, International Commercial Operations at Vertex. “Our medicines have fundamentally changed the way CF is treated and we share the community’s sense of urgency for rapid access. This contract also allows Danes with CF to be among the first in the world to access our future CFTR modulator medicines.”

“I would like to thank Vertex for its will and engagement to make this deal. Most of all, I’m happy that we have made it possible to offer a group of patients the newest treatments. And naturally, I’m also enthusiastic that this new kind of contract gives the Danish hospitals and their owners in the regions much more predictable budgets – at times of rising costs of hospital medicines,” said Flemming Sonne, CEO at Amgros.

Earlier this month, an agreement in Austria was also secured to provide access to ORKAMBI® (lumacaftor/ivacaftor) for all children with CF ages 6 through 11 with two copies of the *F508del* mutation. This agreement is also effective from today.

Austrians and Danes with CF join patients in other countries around the world who have access to CFTR modulator treatments, including Australia, Germany, Ireland, Italy, the Netherlands, Sweden and the U.S.

About CF

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

CF is caused by a defective or missing cystic fibrosis transmembrane conductance regulator (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, or genotyping test, lead to CF by creating non-working or too few CFTR proteins at the cell surface. The defective function or absence of CFTR protein results in poor flow of salt and water into and out of the cell in a number of organs. In the lungs, this leads to the build-up 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 age of death is in the mid-to-late 20s.

About ORKAMBI® (lumacaftor/ivacaftor) and the *F508del* mutation

In people with two copies of the *F508del* mutation, the CFTR protein is not processed and trafficked normally within the cell, resulting in little-to-no CFTR protein at the cell surface. Patients with two copies of the *F508del* mutation are easily identified by a simple genetic test.

ORKAMBI® is a combination of lumacaftor, which is designed to increase the amount of mature protein at the cell surface by targeting the processing and trafficking defect of the F508del-CFTR protein, and ivacaftor, which is designed to enhance the function of the CFTR protein once it reaches the cell surface. Lumacaftor/ivacaftor is available as tablets and is typically taken twice per day.

For complete product information, please see the Summary of Product Characteristics that can be found on www.ema.europa.eu.

About Vertex

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious and life-threatening diseases. In addition to clinical development programs in CF, Vertex has more than a dozen ongoing research programs focused on the underlying mechanisms of other serious diseases.

Founded in 1989 in Cambridge, Mass., Vertex's headquarters is now located in Boston'sInnovation District. Today, the company has research and development sites and commercial offices in the United States, Europe, Canada, Australia and Brazil. Vertex is consistently recognized as one of the industry's top places to work, including being named to Science magazine's Top Employers in the life sciences ranking for eight years in a row.

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, the statements in the second and third paragraphs of this press release. 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, risks related to commercializing our products and the other risks listed under Risk Factors in Vertex's annual report and quarterly reports filed with the Securities and Exchange Commission. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

(VRTX-GEN)

View source version on businesswire.com: https://www.businesswire.com/news/home/20181001005495/en/

Source: Vertex Pharmaceuticals (Europe) Limited

Vertex Pharmaceuticals Incorporated
Investors:
Michael Partridge, +1-617-341-6108
or
Eric Rojas, +1-617-961-7205
or
Zach Barber, +1-617-341-6470
or

Media:
mediainfo@vrtx.com
or
North America:
Heather Nichols, + 1-617-341-6992

or
Europe & Australia:
Rob Clark, +44 7736 473 227