

Vertex Announces Provisional Agreement with Pharmac for the reimbursement of TRIKAFTA® (elexacaftor/tezacaftor/ivacaftor and ivacaftor) for Patients With Cystic Fibrosis Ages 6 Years and Older With at Least One F508del Mutation in the CFTR Gene

Under this agreement, more than 360 New Zealanders with cystic fibrosis will have funded access to Trikafta, including around 340 who will have reimbursed access to a CFTR modulator therapy for the first time –

Wellington – 4 November 2022 - Vertex Pharmaceuticals today announced that it has reached a provisional agreement with New Zealand's medicines purchasing agency, Pharmac, for the reimbursement of TRIKAFTA[®] (elexacaftor/tezacaftor/ivacaftor and ivacaftor). Following a public consultation period, the agreement will cover eligible cystic fibrosis (CF) patients 6 years and older who have at least one *F508del* mutation in the *cystic fibrosis transmembrane* conductance regulator (CFTR) gene with access planned from 1 April 2023.

"Today's announcement is a significant milestone in ensuring New Zealanders living with cystic fibrosis receive timely and sustainable access to Trikafta, including children as young as six," said Sabrina Barbic, Senior Country Manager, Vertex Australia and New Zealand. "We are pleased that Pharmac has recognised that every eligible patient should have access and acknowledges the value Trikafta can bring, not only to people living with cystic fibrosis and their caregivers, but also the wider society."

Pharmac will now seek public consultation on its proposal to fund Trikafta. We acknowledge this as an important step in the process and are hopeful that patients will have access to Trikafta as soon as possible.

On finalisation of the process, New Zealand will join more than thirty-five other countries - including Australia, Canada, Denmark, Finland, Spain, Germany, Austria, Slovenia, Croatia, France, Italy, and the United Kingdom - where Trikafta is broadly reimbursed for eligible CF patients.

About TRIKAFTA® (elexacaftor/tezacaftor/ivacaftor and ivacaftor)

In people with certain types of mutations in the CFTR gene, the CFTR protein is not processed or folded normally within the cell, and this can prevent the CFTR protein from reaching the cell surface and functioning properly. TRIKAFTA® (elexacaftor/tezacaftor/ivacaftor and ivacaftor) is an oral medicine designed to increase the quantity and function of the CFTR protein at the cell surface. Elexacaftor and tezacaftor work together to increase the amount of mature protein at the cell surface by binding to different sites on the CFTR protein. Ivacaftor, which is known as a CFTR potentiator, is designed to facilitate the ability of CFTR proteins to transport salt and water across the cell membrane. The combined actions of elexacaftor, tezacaftor and ivacaftor help hydrate and clear mucus from the airways.

About TRIKAFTA® (elexacaftor/tezacaftor/ivacaftor and ivacaftor) in New Zealand

TRIKAFTA® (elexacaftor/tezacaftor/ivacaftor and ivacaftor) is a prescription medicine approved for the treatment of cystic fibrosis (CF) in patients ages 6 years and older who have at least one copy of the *F508del* mutation in the *CFTR* gene. TRIKAFTA has risks and benefits. Use strictly as directed. TRIKAFTA is currently an unfunded medicine - a prescription charge will apply.

- Consumer Medicine information can be obtained from the Medsafe website www.medsafe.govt.nz
- Prescribing Information (TRIKAFTA New Zealand Data Sheet) for healthcare professionals can be obtained from the Medsafe website www.medsafe.govt.nz

About Cystic Fibrosis

Cystic fibrosis (CF) is a rare, life-shortening genetic disease affecting more than 80,000 people globally. CF is a progressive, multi-system disease that affects the lungs, liver, GI tract, sinuses, sweat glands, pancreas and reproductive tract. CF is caused by a defective and/or missing CFTR protein resulting from certain mutations in the *CFTR* gene. Children must inherit two defective *CFTR* genes — one from each parent — to have CF. While there are many different types of *CFTR* mutations that can cause the disease, the vast majority of all people with CF have at least one *F508del* mutation. These mutations, which can be determined by a genetic test, or genotyping test, lead to CF by creating non-working and/or too few CFTR proteins at the cell surface. The defective function and/or absence of CFTR protein results in poor flow of salt and water into and out of the cells in a number of organs. In 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 age of death is in the early 30s.

About Vertex

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious diseases. The company has multiple approved medicines that treat the underlying cause of cystic fibrosis (CF) — a rare, life-threatening genetic disease — and has several ongoing clinical and research programs in CF. Beyond CF, Vertex has a robust pipeline of investigational small molecule, cell and genetic therapies in other serious diseases where it has deep insight into causal human biology, including sickle cell disease, beta thalassemia, APOL1-mediated kidney disease, pain, type 1 diabetes, alpha-1 antitrypsin deficiency and Duchenne muscular dystrophy.

Founded in 1989 in Cambridge, Mass., Vertex's global headquarters is now located in Boston's Innovation District and its international headquarters is in London. Additionally, the company has research and development sites and commercial offices in North America, Europe, Australia and Latin America. Vertex is consistently recognized as one of the industry's top places to work, including 13 consecutive years on Science magazine's Top Employers list and one of Fortune's Best Workplaces in Biotechnology and Pharmaceuticals and Best Workplaces for Women.