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ASIA EDITION

# Vertex receives approval for CF drug in Australia

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**A new treatment option for patients with two copies of the F508del mutation, the most common mutation in cystic fibrosis**

Vertex Pharmaceuticals Incorporated has announced that the Therapeutic Goods Administration (TGA) of Australia has granted registration to SYMDEKO® (tezacaftor/ivacaftor and ivacaftor) for the treatment of people with cystic fibrosis (CF) aged 12 years and older who are homozygous for the F508del mutation or who have at least one mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene that is responsive to tezacaftor/ivacaftor based on in vitro data and/or clinical evidence.

The TGA's decision is based on results from two pivotal Phase 3 studies, EVOLVE and EXPAND, published in the New England Journal of Medicine in November 2017. Results showed treatment with tezacaftor/ivacaftor in combination with ivacaftor provides benefits across different CF populations, including statistically significant improvements in lung function, as determined by absolute change from baseline in percent predicted forced expiratory volume in one second (ppFEV1), with a generally well tolerated safety profile and a lack of increased respiratory adverse events compared to placebo.

Tezacaftor/ivacaftor in combination with ivacaftor was approved by the U.S. Food and Drug Administration (FDA) in February 2018, by Health Canada in June 2018 and by the European Commission in October 2018.