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Generic Name:

Ivacaftor

Trade Name:

Kalydeco

Company:

Vertex

Notes:

[Vertex](#) announced the FDA approval of [ivacaftor](#), the first medication used to treat the underlying cause of cystic fibrosis (CF). Ivacaftor is approved for people 6 years and older with CF who have at least one copy of the G551D mutation in the *CFTR* (cystic fibrosis transmembrane conductance regulator) gene. Ivacaftor works by targeting abnormal chloride trafficking, the underlying cause of CF. The medication will begin shipping to pharmacies this week.

Approval was based on data from two 48-week, Phase III studies of patients with CF and at least one copy of the G551D mutation. Those who were treated with ivacaftor experienced significant and sustained improvements in lung function and other disease measures, including weight gain and certain quality of life measurements, compared with those who received placebo. In addition, patients treated with ivacaftor experienced significantly fewer pulmonary exacerbations compared with placebo.

Adverse events were generally mild to moderate in nature. Those most commonly observed with ivacaftor included headache, upper respiratory tract infection, stomach pain, and diarrhea.

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Use:

Management of cystic fibrosis

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