Generic Name:
Lumacaftor/ivacaftor
Trade Name:
Orkambi
Company:
Vertex Pharmaceuticals
Notes:

Vertex Pharmaceuticals announced FDA approval of lumacaftor/ivacaftor to include use in children aged 2 through 5 years with cystic fibrosis (CF) who have two copies of the F508del-CFTR mutation, making it the first medication approved to treat the underlying cause of CF in this population.

The oral granules are available in two dosage strengths?lumacaftor 100mg/ivacaftor 125mg and lumacaftor 150mg/ivacaftor 188mg?for weight-based dosing.

FDA approval for this indication was based on a Phase III open-label safety study in 60 patients that showed treatment with lumacaftor/ivacaftor was generally safe and well tolerated for 24 weeks, with a safety profile similar to that in patients aged 6 years and older. Improvements in sweat chloride, a secondary endpoint, were observed at week 24. Researchers also saw changes in key growth parameters, which were also secondary endpoints in the study.

The most common adverse event (?30%) was cough (63%); most adverse events were mild or moderate in severity. Four patients experienced serious adverse events (two pulmonary exacerbations, one gastroenteritis, one constipation), and three patients discontinued treatment due to treatment-emergent adverse events or elevated liver function tests.

The agent was originally approved for treatment of CF in patients aged 6 years and older who have two copies of the F508del-CFTR mutation.

Medication Monitor Categories:
Supplemental Approvals