

(<http://aphanet.org>)

[Home](#) > First agent to target underlying cause of CF in children as young as 12 months

Generic Name:

Ivacaftor

Trade Name:

Kalydeco

Company:

Vertex Pharmaceuticals

Notes:

[Vertex Pharmaceuticals](#) announced FDA approval of ivacaftor to include use in children with cystic fibrosis (CF) aged 12 months to younger than 24 months who have at least one mutation in their cystic fibrosis transmembrane conductance regulator (CFTR) gene that is responsive to ivacaftor, based on clinical and/or in vitro assay data.

FDA approval for this indication was based on data from the ongoing Phase III open-label safety study (ARRIVAL) of 25 children with CF aged 12 months to younger than 24 months who have 1 of 10 mutations in the CFTR gene.

The study demonstrated a safety profile consistent with that observed in previous Phase III studies of older children and adults; most adverse events were mild or moderate in severity, and no patient discontinued because of adverse events. Two patients had elevated liver enzymes greater than eight times the upper limit of normal but continued to receive ivacaftor after a dose interruption.

The most common adverse events (?30%) were cough (74%), pyrexia (37%), elevated aspartate aminotransferase (37%), elevated alanine aminotransferase (32%), and runny nose (32%). Four serious adverse events were observed in two patients.

Mean baseline sweat chloride for the children in this study was 104.1 mmol/L (n = 14). Following 24 weeks of treatment with ivacaftor, the mean sweat chloride level was 33.8 mmol/L (n = 14). In the 10 participants with paired sweat chloride samples at baseline and week 24, there was a mean absolute change of -73.5 mmol/L.

Ivacaftor was previously approved for treatment of CF in patients aged 2 years and older who have 1 of 38 ivacaftor-responsive mutations in the CFTR gene, based on clinical and/or in vitro assay data.

Medication Monitor Categories:

[Supplemental Approvals](#)

Source URL: <http://aphanet.org/supplemental-approvals/first-agent-target-underlying-cause-cf-children-young-12-months>