

primary studies - published RCT

Long-term safety and efficacy of ivacaftor in patients with cystic fibrosis who have the Gly551Asp-CFTR mutation: a phase 3, open-label extension study (PERSIST).

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Study design (if review, criteria of inclusion for studies)

Phase 3, open-label extension study

Participants

Patients with cystic fibrosis who have the Gly551Asp-CFTR mutation on at least one allele. 144 adolescents/adults (>/=12 years) from STRIVE and 48 children (6-11 years) from ENVISION were enrolled.

Interventions

Patients received ivacaftor 150 mg every 12 h in addition to their prescribed cystic fibrosis therapies. Patients who received placebo in their previous study initiated ivacaftor in this extension study.

Outcome measures

Long-term safety as assessed by adverse events, clinical laboratory assessments, electrocardiograms, vital signs, and physical examination; secondary measures included change in forced expiratory volume in one second (FEV1), weight, and pulmonary exacerbations.

Main results

Between July 8, 2010, and April 8, 2013, 144 adolescents/adults (>/=12 years) from STRIVE and 48 children (6-11 years) from ENVISION were enrolled. Across both trials, 38 (20%) patients had a serious adverse event during the first 48 weeks and 44 (23%) during the subsequent 48 weeks. Two adults (1%) and one child (

Authors' conclusions

At 144 weeks of treatment, ivacaftor was well tolerated, with no new safety concerns. Ivacaftor also provided durable effects for 144 weeks in patients who had received active treatment in the placebo-controlled study. Those patients who previously received placebo had improvements comparable to those of patients treated with ivacaftor in the placebo-controlled study.

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See also

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Keywords

Adult; Aged; Child; Cystic Fibrosis Transmembrane Conductance Regulator; pharmacological_intervention; VX-770; ivacaftor; Aminophenols;