primary studies - published RCT

Long-Term Safety and Efficacy of Elexacaftor/Tezacaftor/Ivacaftor inÂ Children Aged â©¾6 Years with Cystic Fibrosis and at Least One F508del Allele: A Phase 3, Open-Label Clinical Trial.

Code: PM37154609 **Year:** 2023 **Date:** **Author:** Wainwright C

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

Phase 3, two-part (part A and part B), open-label extension study

Participants

Children Aged â©¾6 Years with Cystic Fibrosis and at Least One F508del Allele

Interventions

Children weighing

Outcome measures

The primary endpoint was safety and tolerability. Adverse events and serious adverse events were consistent with common manifestations of CF disease.

Main results

Overall, exposure-adjusted rates of adverse events andÂ serious adverse events (407.74 and 4.72 events per 100 patient-years) were lower than in the parent study (987.04Â andÂ 8.68 events per 100 patient-years). One child (1.6%) had an adverse event of aggression that was moderate in severity and resolved after study drug discontinuation. From parent study baseline at Week 96 of this extension study, the mean percent predicted FEV(1) increased (11.2Â [95% confidence interval (CI), 8.3 to 14.2] percentage points), sweat chloride concentration decreased (-62.3 [95% CI, -65.9 to -58.8] mmol/L), Cystic Fibrosis Questionnaire-Revised respiratory domain score increased (13.3 [95% CI, 11.4 to 15.1] points), and lung clearance index 2.5 decreased (-2.00 [95% CI, -2.45 to -1.55] units). Increases in growth parameters were also observed. The estimated pulmonary exacerbation rate per 48â€‰weeks was 0.04. The annualized rate of change in percent predicted FEV(1) was 0.51 (95% CI, -0.73 to 1.75) percentage points per year.

Authors' conclusions

ELX/TEZ/IVA continued to be generally safe and well tolerated in children aged â©¾6â€‰years through an additional 96â€‰weeks of treatment. Improvements in lung function, respiratory symptoms, and CFTR function observed in the parent study were maintained. These results demonstrate the favorable long-term safety profile and durable clinical benefits of ELX/TEZ/IVA in this pediatric population.

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See also

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Keywords

CFTR Modulators; Genetic Predisposition to Disease; pharmacological_intervention; placebo; VX-770; VX-661; ivacaftor; Aminophenols; tezacaftor; VX-445; elexacaftor; Trikafta; Child;