primary studies - published RCT

Elexacaftor/tezacaftor/ivacaftor in children aged â‰¥6â€…years with cystic fibrosis heterozygous for F508del and a minimal function mutation: Results from a 96-week open-label extension study.

Code: PM40210412 **Year:** 2025 **Date:** **Author:** Mall MA

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

Phase 3b extension study

Participants

Children aged 6-11â€…years with cystic fibrosis (CF) heterozygous for F508del and a minimal function CFTR variant (F/MF genotypes)

Interventions

Dosing was based on weight and age with children weighing

Outcome measures

Primary endpoint was safety and tolerability. Secondary and other efficacy endpoints included absolute changes from parent study baseline in sweat chloride concentration, LCI(2.5), ppFEV(1), and CFQ-R respiratory domain score.

Main results

A total of 120 children were enrolled and dosed. One hundred and eighteen children (98.3%) had adverse events (AEs), which for most were mild (43.3%) or moderate (48.3%) in severity. The most common AEs (â‰¥20% of children) were COVID-19 (58.3%), cough (51.7%), nasopharyngitis (45.0%), pyrexia (40.0%), headache (37.5%), upper respiratory tract infection (30.8%), oropharyngeal pain (26.7%), rhinitis (24.2%), abdominal pain (22.5%), and vomiting (20.0%). Children who transitioned from the placebo and ELX/TEZ/IVA groups of the parent study had improvements from parent study baseline at Week 96 in mean sweat chloride concentration (-57.3 [95% CI: -61.6, -52.9] and -57.5 [95% CI: -62.0, -53.0] mmolÂ·L(-1)), LCI(2..5) (-1.74 [95% CI: -2.09, -1.38] and -2.35 [95% CI: -2.72, -1.97] units), ppFEV(1) (6.1 [95% CI: 2.6, 9.7] and 6.9 [95% CI: 3.2, 10.5] percentage points), and CFQ-R respiratory domain score (6.6 [95% CI: 2.5, 10.8] and 2.6 [95% CI: -1.6, 6.8] points).

Authors' conclusions

ELX/TEZ/IVA treatment was generally safe and well-tolerated, with a safety profile consistent with parent study and older age groups. After starting ELX/TEZ/IVA, children had robust improvements in sweat chloride concentration and lung function that were maintained through 96â€…weeks. These results demonstrate the safety and durable efficacy 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;