primary studies - published RCT

Effects of Lumacaftor/Ivacaftor on Cystic Fibrosis Disease Progression in Children 2 through 5 Years of Age Homozygous for F508del-CFTR: A Phase 2 Placebo-controlled Clinical Trial.

Code: PM36943405 **Year:** 2023 **Date:** 2023 **Author:** Stahl M

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

Phase 2 study. Two parts: a 48-week, randomized, double-blind, placebo-controlled treatment period (Part 1) followed by an open-label period

Participants

Children 2 through 5 years of age with CF homozygous for F508del-CFTR

Interventions

This study had two parts: a 48-week, randomized, double-blind, placebo-controlled treatment period in which children 2 through 5 years of age with CF homozygous for F508del-CFTR received either LUM/IVA or placebo (Part 1) followed by an open-label period in which all children received LUM/IVA for an additional 48 weeks (Part 2).

Outcome measures

The primary endpoint was absolute change from baseline in chest MRI global score at Week 48. Secondary endpoints included absolute change in LCI(2.5) through Week 48 and absolute changes in weight-for-age, stature-for-age, and body mass index-for-age z-scores at Week 48. Additional endpoints included absolute changes in sweat chloride concentration, fecal elastase-1 levels, serum immunoreactive trypsinogen, and fecal calprotectin through Week 48.

Main results

Authors report results from Part 1. Fifty-one children were enrolled and received LUM/IVA (n=35) or placebo (n=16). For the change in MRI global chest score at Week 48, the Bayesian posterior probability of LUM/IVA being better than placebo (treatment difference

Authors' conclusions

This placebo-controlled study suggests the potential for early disease modification with LUM/IVA treatment, including that assessed by chest MRI, in children as young as 2 years.

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See also

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Keywords

Child; Aminophenols; CFTR Modulators; Genetic Predisposition to Disease; Orkambi; pharmacological_intervention; VX-770; ivacaftor; lumacaftor; VX-809;