primary studies - published RCT

Riociguat for the treatment of Phe508del homozygous adults with cystic fibrosis.

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

Multicenter, two-part, Phase II study. Part 1 was a 28-day, randomized, double-blind, placebo-controlled study

Participants

Adults with cystic fibrosis (CF) homozygous for Phe508del CFTR.

Interventions

Riociguat, a first-in-class soluble guanylate cyclase stimulator for which preclinical data suggested improvements in cystic fibrosis transmembrane conductance regulator (CFTR) function. Twenty-one participants were randomized 1:2 to placebo or oral riociguat (0.5 mg three times daily [tid] for 14 days, increased to 1.0 mg tid for the subsequent 14 days).

Outcome measures

The primary and secondary efficacy endpoints were change in sweat chloride concentration and percent predicted forced expiratory volume in 1 second (ppFEV(1)), respectively, from baseline to Day 14 and Day 28 with riociguat compared with placebo.

Main results

Riociguat did not alter CFTR activity (change in sweat chloride) or lung function (change in ppFEV(1)) at doses up to 1.0 mg tid after 28 days. The most common drug-related adverse event (AE) was headache occurring in three participants (21%); serious AEs occurred in one participant receiving riociguat (7%) and one participant receiving placebo (14%). This safety profile was consistent with the underlying disease and the known safety of riociguat for its approved indications.

Authors' conclusions

The Rio-CF study was terminated due to lack of efficacy and the changing landscape of CF therapeutic development. The current studyâ• , within its limits of a small sample size, did not provide evidence that riociguat could be a valid treatment option for CF.

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See also

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Keywords

Adempas; Riociguat; Respiratory System Agents; pharmacological_intervention;