primary studies - published RCT

Efficacy and safety of LAU-7b in a Phase 2 trial in adults with cystic fibrosis.

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

Double-blind, randomized, placebo-controlled Phase 2 study

Participants

Adults with CF.

Interventions

LAU-7b, a novel oral drug candidate. LAU-7b or placebo was administered over 24 weeks as six 21-day treatment cycles each separated by 7 days.

Outcome measures

The primary efficacy endpoint was the absolute change from baseline in percent predicted forced expiratory volume in 1 second (ppFEV(1)) at 24 weeks.

Main results

A total of 166 subjects received at least one dose of study drug (Intent-To-Treat population, ITT), of which 122 received â‰¥5 treatment cycles (Per-Protocol population, PP). Both treatment arms showed a mean lung function loss at 24 weeks of 1.18 ppFEV(1) points with LAU-7b and 1.95 ppFEV(1) with placebo, a 0.77 ppFEV(1) (40 s) difference, p=0.345, and a 0.95 ppFEV(1) (49 %) difference in the same direction in PP population, p=0.263. Primary analysis of mean ppFEV(1) through 24 weeks showed differences of 1.01 and 1.23 ppFEV(1), in the ITT (65 % less loss, p=0.067) and PP populations (78 % less loss, reaching statistical significance p=0.049), respectively. LAU-7b had an acceptable safety profile.

Authors' conclusions

Although the study did not meet its primary efficacy endpoint in the ITT population, LAU-7b was generally well tolerated and showed evidence of preservation of lung function to support further development.

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See also

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Keywords

Fenretinide; Anti-Inflammatory Agents - excl Steroids; pharmacological_intervention;