

primary studies - published RCT

# **Efficacy and safety of ataluren in patients with nonsense-mutation cystic fibrosis not receiving chronic inhaled aminoglycosides: The international, randomized, double-blind, placebo-controlled Ataluren Confirmatory Trial in Cystic Fibrosis (ACT CF).**

**Code:** PM31983658

**Year:** 2020 **Date:**

**Author:** Konstan MW

## **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  $\approx 5$  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.

<http://dx.doi.org/10.1016/j.jcf.2020.01.007>

## **See also**

J Cyst Fibros. 2020 Jan 23. pii: S1569-1993(20)30030-8. doi: 10.1016/j.jcf.2020.01.007.

## **Keywords**

Fenretinide; Anti-Inflammatory Agents - excl Steroids; pharmacological\_intervention;