

primary studies - published RCT

## **Elexacaftor-Tezacaftor-Ivacaftor for Cystic Fibrosis with a Single Phe508del Allele.**

**Code:** PM31697873

**Year:** 2019 **Date:** 2019

**Author:** Middleton PG

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

Phase 3, randomized, double-blind, placebo-controlled trial

### **Participants**

Patients 12 years of age or older with cystic fibrosis with Phe508del-minimal function genotypes.

### **Interventions**

Patients were randomly assigned to receive elexacaftor-tezacaftor-ivacaftor or placebo for 24 weeks.

### **Outcome measures**

The primary end point was absolute change from baseline in percentage of predicted forced expiratory volume in 1 second (FEV1) at week 4.

### **Main results**

A total of 403 patients underwent randomization and received at least one dose of active treatment or placebo. Elexacaftor-tezacaftor-ivacaftor, relative to placebo, resulted in a percentage of predicted FEV1 that was 13.8 points higher at 4 weeks and 14.3 points higher through 24 weeks, a rate of pulmonary exacerbations that was 63% lower, a respiratory domain score on the Cystic Fibrosis Questionnaire-Revised (range, 0 to 100, with higher scores indicating a higher patient-reported quality of life with regard to respiratory symptoms; minimum clinically important difference, 4 points) that was 20.2 points higher, and a sweat chloride concentration that was 41.8 mmol per liter lower (P

### **Authors' conclusions**

Elexacaftor-tezacaftor-ivacaftor was efficacious in patients with cystic fibrosis with Phe508del-minimal function genotypes, in whom previous CFTR modulator regimens were ineffective.

<http://dx.doi.org/10.1056/NEJMoa1908639>

### **See also**

N Engl J Med. 2019 Nov 7;381(19):1809-1819. doi: 10.1056/NEJMoa1908639. Epub 2019 Oct 31.

### **Keywords**

Adult; Aged; CFTR Modulators; Genetic Predisposition to Disease; pharmacological\_intervention; placebo; VX-770; VX-661; ivacaftor; Aminophenols; tezacaftor; VX-445; elexacaftor; Trikafta;