

# Final results Choices HIT-CF trial: clinical effect proportional to in-vitro effect

UTRECHT, NETHERLANDS, October 20, 2025 /EINPresswire.com/ -- [The HIT-CF Consortium](#) presented its final results on the evaluation of new CFTR-modulator drugs to the European Commission which funded the project under the European Union's Horizon 2020 research and innovation program (755021).

The clinical effect of the CFTR modulator combination Dirocaftor-Posenacaftor-Nesolicaftor, as licensed [by Fair Therapeutics](#) was proportional to the in vitro effects in people with cystic fibrosis (PwCF) with all classes of CFTR gene (and ultra rare) variants. In a placebo-controlled double blinded study, changes in percentages predicted FEV1 ranged from -12% up to +22% percentage points with concordant changes in sweat chloride levels and patient reported outcomes (CFQ-R). In vitro responses were measured in organoids of over 500 pwCF from 16 different European countries using the forskolin induced swelling (FIS) assay.

The results strongly underline the earlier supporting statement of the European Medicines Agency that the FIS assay is a reproducible ex vivo biomarker with strong biological rationale. They also support the use of FIS to: 1) pre-select individuals for clinical trials; and 2) select responders in vitro among PwCF with rare variants not considered in commercial clinical trials with CFTR modulators.

The study also shows promising clinical effects of the new CFTR modulator combination Dirocaftor-Posenacaftor-Nesolicaftor of Fair Therapeutics as first-line treatment in PwCF who show responsiveness in the FIS assay, with a favorable safety profile.

The European Commission strongly supports the further development of both biomarker-driven (individualized) prescription of disease-modifying drugs in chronic diseases in general through cost-effective precision medicine approaches, as well as the further development of the new CFTR modulator combination for PwCF worldwide.

Professor Dr Kors van der Ent, coordinator of the HIT-CF project said: 'We are really thrilled about the results of our project. Despite all hurdles during the pandemic and changing industry partners, the entire European CF community showed perseverance and dedication to realize new treatments for PwCF.'

Mrs Anne van Loon, CEO of Fair Therapeutics stated: 'FAIR Therapeutics is proud to show the

results of their new modulator combination. Around 45,000 diagnosed people with CF do not have access to a potentially life-changing treatment. We are determined to set next steps in the development of this combination and complete our mission to transform CF treatment.'

Mrs Hilde De Keyser, CEO of CF Europe, the federation of national and regional patient associations of PwCF in Europe mentioned: 'The patient community was actively involved in the HIT-CF project and we strongly support the goal to realize treatment for patients with ultra-rare mutations and in underserved areas.'

The HIT-CF Consortium consists of the University Medical Center Utrecht (The Netherlands) (coordinator); the KU Leuven, Belgium; Faculdade de Ciências da Universidade de Lisboa, Portugal; The European Cystic Fibrosis Society, Aarhus (Denmark), CF Europe, Fair Therapeutics B.V., Utrecht (The Netherlands), HUB Organoids, Utrecht (The Netherlands), Julius Clinical, Utrecht (The Netherlands), and Patergus – Biotechsubsidy, Aalter, Belgium.

Fair Therapeutics (Fair TX) is a privately held, clinical-stage biopharmaceutical company developing accessible, personalized treatments for Cystic Fibrosis.

Cystic Fibrosis is a genetic life-shortening disorder affecting the lungs, digestive system, and other organs. It is caused by variants in the CFTR gene, leading to thick, sticky mucus buildup. This clogs airways, traps bacteria, leading to progressive loss of lung function and also disrupts digestion. Without CFTR modulator therapies median survival is limited to early adulthood.

Anne van Loon  
Fair Therapeutics  
info@fairtherapeutics.com

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