

Press Release

Fair Therapeutics B.V. Announces Full Enrollment in Phase IIb CHOICES Trial Evaluating Novel CFTR Modulator Therapy for People with Cystic Fibrosis with Ultra-Rare Variants

Utrecht, The Netherlands, 8 January 2025 – Fair Therapeutics B.V. (Fair TX), a clinical-stage biopharmaceutical company focused on personalized treatments for cystic fibrosis (CF), today announced the full enrollment of its Phase IIb clinical trial, CHOICES (NCT06468527). The trial aims to assess the efficacy and safety of Fair TX's novel Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) triple modulator therapy—Diponecaftor (Dirocaftor/Posenacaftor/Nesolicaftor)—in adults with ultra-rare CF variants who currently cannot benefit from any disease-modifying treatment. The CHOICES trial is a pivotal step in the company's mission to address the high unmet needs of people with cystic fibrosis (PwCF). Results of the trial are expected mid-2025.

The trial, a double-blinded cross-over trial, began in June 2024, and has enrolled 40 adult patients (18+) from 9 European countries. All participants were pre-selected using the innovative organoid test, which uses laboratory-grown organoids from patients' own cells to predict clinical response to the therapy in the lab.

Key objectives of the CHOICES trial include:

- **Evaluating efficacy:** The trial will assess the predicted clinical impact of Fair TX's CFTR modulators in PwCF identified through their organoid response.
- **Focusing on ultra-rare variants:** The trial aims to demonstrate that Diponecaftor is effective in treating ultra-rare CF variants, a patient population with limited treatment options.
- **Validating the organoid model:** Data from the trial will be compared with results from the earlier Human Individualized Therapy of CF (HIT-CF) Organoid Study (NTR7520), which tested CFTR modulators on organoids from over 500 European PwCF with rare variants. This comparison will further validate the use of organoids as a reliable predictive tool to preselect PwCF for the treatment.

The CHOICES trial is being executed by the HIT-CF Europe consortium, a collaborative effort supported by the EU Horizon 2020 initiative. The consortium includes academic research institutions, not-for-profit organizations, contract research organizations (CROs), biopharma companies developing CF therapies, and supporting service providers ([click here](#) for the full list of consortium partners). Fair TX's Co-Founder, Dr. Kors van der Ent, chairs the HIT-CF Europe consortium, playing a pivotal role in leading the trial.

Dr. Kors van der Ent, Co-Founder of Fair TX, commented:

"We are thrilled to have completed enrollment for the CHOICES trial, which represents a significant milestone for Fair TX and the cystic fibrosis community. By focusing on ultra-rare CF variants and using organoid-based diagnostics, we are advancing personalized medicine and bringing much-needed hope to PwCF who have had limited or no treatment options. We look forward to sharing the results of this groundbreaking trial."

Anne van Loon, Acting CEO of Fair TX, added:

"Completing enrollment in the CHOICES trial highlights our commitment to addressing the unmet needs of people with ultra-rare CF variants. This is an exciting time for Fair TX as we continue to demonstrate the potential of our personalized approach to treatment. We are deeply grateful to the patients, families, and clinical teams who have made this milestone possible."

The Phase IIb CHOICES trial is being conducted at 15 clinical centers across Europe. The final patient has now been enrolled, and all 40 participants have commenced treatment.

For more information on the HIT-CF project, please visit <https://www.hitcf.org>.

About Fair Therapeutics

Fair Therapeutics (Fair TX) is a privately held, clinical-stage biopharmaceutical company developing personalized treatments for cystic fibrosis (CF). Diponecaftor, the company's innovative CFTR triple modulator therapy, combined with its companion diagnostic based on laboratory-grown organoids, aims to address the unmet needs of PwCF with rare and ultra-rare variants. Committed to fair medicine principles, Fair TX strives to ensure fair value and equal access to treatment. Founded in 2021, the company collaborates with industry, academic research institutions, healthcare professionals, and patient advocacy groups to advance its mission.

For more information, visit www.fairtherapeutics.com or contact:

Media Contact:

Anne van Loon

Acting CEO

+31 683 087 289

info@fairtherapeutics.com