PRESS RELEASE
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Enterprise Therapeutics publishes results of Phase 1 study of ETD001, a novel inhaled ENaC blocker for treatment of Cystic Fibrosis, in The Journal of Cystic Fibrosis

- *ETD001 is well tolerated in healthy individuals at higher doses than predicted to be therapeutic for long-lasting enhancement of mucociliary clearance*
- *Results showed excellent consistency between preclinical and clinical behaviour of ETD001*
- *Phase 2 trial of ETD001 in people with cystic fibrosis is in progress with headline data anticipated early 2026*

Brighton, UK, 09 December 2025: Enterprise Therapeutics Ltd (Enterprise), a biopharmaceutical company dedicated to the discovery and development of novel therapies to improve the lives of people with respiratory diseases, today announced the publication of a peer reviewed study in The Journal of Cystic Fibrsosis[1]. The paper, titled '*ETD001, a long-acting inhaled ENaC blocker, is well tolerated in a first human, healthy participant trial*', describes the results of the Phase 1 trial of Enterprise's novel epithelial sodium channel (ENaC) blocker, ETD001, indicating that ETD001 is well tolerated in healthy individuals at higher doses than required to observe long-lasting improvements in mucociliary clearance in preclinical models.

The Phase 1 study to evaluate the safety, tolerability and pharmacokinetics of ETD001 followed single and multiple inhaled ascending doses, at levels predicted to be efficacious in humans. The results from the trial show that ETD001 was well tolerated at single doses and on repeat dosing twice daily, for up to 14 days. Unlike historical inhaled ENaC blockers, the pharmacokinetic profile of ETD001 following inhaled dosing was consistent with slow absorption out of the lung and into the systemic circulation, consistent with prolonged retention in the lung and the potential for extended duration of action. Furthermore, target-mediated ENaC blockade in the kidney can lead to changes in blood potassium levels. This trial demonstrated that levels of blood potassium remained within normal limits at all doses of ETD001 evaluated.

These results align exceptionally well with the pre-clinical data[2], which demonstrated a good safety profile and an extended duration of action in the lung for >16 hours after a single inhaled dose. Enterprise is currently conducting a Phase 2 trial (NCT06478706) investigating whether 28 days of treatment with ETD001 improves lung function in people with cystic fibrosis, with headline data expected in early 2026.

Dr Henry Danahay, Head of Biology, Enterprise Therapeutics, and lead author of the paper, said: "*There is an urgent need for new therapies to treat mucus obstruction in the lungs of people with CF, and especially those who are genetically unsuited to CFTR modulators. We are passionate about working towards treatments that will benefit all people with cystic fibrosis and are excited to publish these promising results from our Phase 1 trial. We are grateful for everyone who took part in this trial, and for the people with cystic fibrosis that are enrolled in the ongoing Phase 2 trial of ETD001.*"

Cystic fibrosis is estimated to affect over 100,000 people worldwide, with an average life expectancy of ~60 years. Failed mucociliary clearance and mucus congestion in the lungs leads to cycles of infection and inflammation and an ongoing decline in lung function. Inhibition of ENaC in the airways of people with cystic fibrosis represents an approach to restore airway mucus hydration and is expected to drive substantial improvements in lung function.

1. *https://www.cysticfibrosisjournal.com/article/S1569-1993(25)02529-9/fulltext*
2. *https://enterprisetherapeutics.com/enterprise-therapeutics-publishes-preclinical-profile-of-etd001-a-novel-inhaled-enac-blocker/*

For more information about Enterprise Therapeutics, visit www.enterprisetherapeutics.com

ENDS

Dr Henry Danahay, Head of Biology, Enterprise Therapeutics

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About Enterprise Therapeutics www.enterprisetherapeutics.com
Enterprise Therapeutics is discovering and developing new therapies that target the underlying mechanisms of mucus congestion in the lungs, one of the main causes of difficulty in breathing and increased risk of infection in respiratory diseases such as cystic fibrosis and COPD. Reducing mucus congestion will reduce the frequency of lung infections and improve patient quality of life.

The Company's approach is to increase the hydration and clearance of mucus. Enterprise's lead asset, ETD001, is a novel inhaled blocker of the epithelial sodium channel (ENaC) with first in class potential. Enterprise has also identified novel targets and compounds that reduce mucus production, an approach that complements mucus hydration therapies.

The Enterprise Therapeutics management team has significant expertise in drug discovery, drug development, respiratory biology and ion channel pharmacology.

About Cystic Fibrosis (CF)
Cystic Fibrosis is a lethal genetic disease, with more than 100,000 patients worldwide living with the disease. The average life expectancy of a person with CF, although improving, is only ~60 years.