Enterprise Therapeutics Granted EMA Orphan Medicinal Product Designation for Novel Cystic Fibrosis Therapy, ETD001

- *Investigational inhaled ENaC blocker developed to treat people with cystic fibrosis not benefitting from CFTR modulators*
- *Positive opinion recognises potential of ETD001 in treating rare and severe disease*
- *Decision follows successful Phase 2 clinical trial*

BRIGHTON, England--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 that the European Medicines Agency (EMA) Committee for Orphan Medicinal Products (COMP) has granted ETD001 designation as an orphan medicinal product for the treatment of cystic fibrosis. This follows the announcement earlier this year of a positive Phase 2 clinical trial outcome of ETD001[1], an investigational inhaled epithelial sodium channel (ENaC) blocker, designed to increase lung function by improving mucus clearance and reducing airway obstruction.

The EMA COMP opinion concluded that ETD001 meets the orphan-designation criteria, recognising disease rarity and the potential for ETD001 to provide clinically relevant benefit, including for people who are not eligible for CFTR modulator therapy. The recommendation was supported by clinical data from the Phase 2 ET-ENAC-03 study, which were indicative of lung function improvement. Orphan medicine designation offers a range of benefits, including market exclusivity once the therapy is on the market.

Dr John Ford, CEO, Enterprise Therapeutics, commented: *"Orphan drug designation represents an important regulatory milestone for the ETD001 programme and for Enterprise Therapeutics. The decision by the EMA reflects the continued need for additional treatment options for people with cystic fibrosis, particularly those who are not able to benefit from CFTR modulators. We look forward to continuing the development of ETD001 in CF as well as other mucus congestion related respiratory diseases."*

Annabella Amatulli, Head of Regulatory Affairs, Enterprise Therapeutics, added: *"Our regulatory strategy supports our global development plan for ETD001, aligned with our goal to provide improved therapeutic options for people with cystic fibrosis. The EMA's orphan*

drug designation further validates the strength of the programme's scientific rationale and outcomes of the Phase 2 clinical trial."

Cystic fibrosis is estimated to affect over 100,000 people worldwide, and leads to reduced life expectancy. Failed mucociliary clearance and mucus congestion in the lungs leads to cycles of infection and inflammation and an ongoing decline in lung function.

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

1. Enterprise Therapeutics achieves primary efficacy outcome in Phase 2 clinical trial for cystic fibrosis with novel inhaled ENaC blocker ETD001 – Enterprise Therapeutics

Contacts

Media contact:
Katie Odgaard,
Zyme Communications
Tel: +44 (0)7787 502 947
Email: katie.odgaard@zymecommunications.com