4D-710 in Adult Patients With Cystic Fibrosis

Status: RECRUITING

Eligibility Criteria

Age: 18 years and over

This study is NOT accepting healthy

Healthy Volunteers: volunteers

Key Inclusion Criteria (Primary Study): 1. 18 years and older 2. Confirmed diagnosis of cystic fibrosis (CF) and CF lung disease including: 1. Sweat chloride ≥ 60 mmol/L 2. Mutation Status * Bi-allelic mutations in the CFTR gene, or * Single mutation in the CFTR gene and clinical manifestations of CF lung disease 3. Ineligible for CFTR modulator therapy, or previously received modulator therapy but discontinued due to adverse effects. 3. Forced expiratory volume in 1 second (FEV1) ≥ 50% and ≤ 90% of predicted (per Global Lung Function Initiative) at Screening 4. Resting oxygen saturation ≥ 92% on room air at Screening Key Inclusion Criteria (Sub-Study): 1. 18 years and older 2. Confirmed diagnosis of cystic fibrosis (CF) and CF lung disease including: 1. Sweat chloride ≥ 60 mmol/L 2. Mutation Status * Bi-allelic mutations in the CFTR gene, or * Single mutation in the CFTR gene and clinical manifestations of CF lung disease 3. Currently on a stable dose of CFTR modulator therapy (elexacaftor/tezacaftor/ivacaftor) for a minimum of 60 days prior to Screening and agree to maintain current regimen through the 12-month Observation Period 4. FEV1 ≥ 40% and \< 70% predicted (per Global Lung Function Initiative) at Screening, AND/OR experienced at least 2 pulmonary exacerbations in the last year requiring intravenous antibiotics Key Exclusion Criteria (Primary and Sub Study): 1. Any prior gene therapy for any indication (Exception: mRNA-based therapies are not exclusionary) 2. Active Mycobacterium abscessus infection requiring ongoing treatment at Screening 3. Active allergic bronchopulmonary aspergillosis requiring management with systemic corticosteroids or antifungal therapy 4. Contraindication to systemic corticosteroid therapy 5. Requires chronic use of systemic corticosteroids or immunosuppressants to treat another condition 6. If no known diagnosis of cystic fibrosis related diabetes (CFRD), Type I, or Type II diabetes: Hemoglobin A1C ≥ 6.5% at Screening 7. If known diagnosis of CFRD, Type I or Type II diabetes: Hemoglobin A1C \> 7.5% at Screening 8. Recent history of symptomatic hyperglycemia or unstable blood glucose levels as per Investigator's assessment 9. Other conditions that, in the Investigator's opinion, may interfere with management of corticosteroid-related hyperglycemia 10. Body Mass Index (BMI) \< 16 11. Laboratory abnormalities at screening: * ALT, AST or GGT ≥ 3 × the upper limit of normal (ULN) * Total bilirubin ≥ 2 × ULN * Hemoglobin \< 10 g/dL 12. Requirement for continuous or night-time oxygen supplementation 13. Known CF liver disease with evidence of multilobular cirrhosis 14. History of thrombosis (excluding catheter-related thrombosis) or conditions associated with increased risk of thrombosis

Conditions & Interventions

Interventions:
BIOLOGICAL: 4D-710

Conditions:
Cystic Fibrosis Lung

Keywords:
CF, Cystic Fibrosis, Gene Therapy

More Information

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Principal Investigator:

Phase: PHASE1

IRB
Number:

System ID: NCT05248230