Study to Evaluate Biological & Clinical Effects of Significantly Corrected CFTR Function in Infants & Young Children

Status: RECRUITING

Eligibility Criteria

Sex: ALL
Age: Up to 10 years old
Healthy Volunteers: This study is NOT accepting healthy volunteers
Inclusion Criteria:

- Part A:
    - Less than 10 years of age at the first study visit.
    - Documentation of a CF diagnosis.

Part B:

- Participated in Part A OR less than 7 years of age at the first study visit.
- Documentation of a CF diagnosis.
- CFTR mutations consistent with FDA labeled indication of highly effective modulator therapy (ivacaftor or elexacaftor/tezacaftor/ivacaftor).
- Physician intent to prescribe ivacaftor or elexacaftor/tezacaftor/ivacaftor.

Exclusion Criteria:

- Part A and Part B:
- Use of an investigational drug within 28 days prior to and including the first study visit.
- Use of ivacaftor or elexacaftor/tezacaftor/ivacaftor within the 28 days prior to and including the first study visit.
- Use of chronic oral corticosteroids within the 28 days prior to and including the first study visit.

Conditions & Interventions

Interventions:
DRUG: Ivacaftor or elexacaftor/tezacaftor/ivacaftor
Conditions:
Cystic Fibrosis
Keywords:
Cystic Fibrosis, CF, CFTR Modulator, triple combination therapy, elexacaftor, tezacaftor, ivacaftor

More Information

Description:
This is a two-part, multi-center, prospective longitudinal, exploratory study of highly effective cystic fibrosis transmembrane conductance regulator (CFTR) modulators and their impact on children with cystic fibrosis (CF).

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System ID: NCT04509050