

Study to evaluate the effects of the triple-combination modulator, elexacaftor/tezacaftor/ivacaftor (PROMISE) (PROMISE-OB-18)

Summary

This observational study will measure the effects of the triple-combination therapy, elexacaftor/tezacaftor/ivacaftor, in people with CF. These drugs are intended to help CFTR protein function closer to normal. This study will look at how treatment with the triple-combination therapy affects people with CF across many different aspects of the disease.

This study is observational. The elexacaftor/tezacaftor/ivacaftor triple-combination therapy will not be prescribed as part of the study. Participants must receive a prescription for the triple-combination therapy from their own CF doctor. Participants will enroll in the study before they begin taking the triple-combination therapy. Researchers will then study changes in sweat chloride, lung function, and other aspects of the participant's health to better understand how treatment with the triple-combination therapy affects different parts of the body. Researchers will also use samples collected during this study to help support future CF research.

This study will require lung function tests, sweat tests, blood draws, physical exams, and urine samples. Other additional procedures may be required at your specific study site. Participants will be required to fast for 8 hours before all study visits.

Specimen Information

Status: Specimens are Not yet available, anticipated April, 2023

HNE cells will be collected once during the study (not visit specific) from those participants who have consented for the HNE sub-study., Due to the COVID-19 pandemic the study was paused and Visits 5 and 6 were delayed by 6 months.

Visit #	Time From Baseline	Specimens Collected
1	+0 Days	Buffy coat, EDTA plasma, Serum, Urine
2	+28 Days	Buffy coat, EDTA plasma, Serum, Urine
3	+3 Months	Buffy coat, EDTA plasma, Serum, Urine
4	+6 Months	Buffy coat, EDTA plasma, Serum, Urine
5	+18 Months	Buffy coat, EDTA plasma, Serum, Urine
6	+30 Months	Buffy coat, EDTA plasma, Serum, Urine

Study Design

Study Type?	Observational
Randomized Study?	No
Placebo Controlled?	No
Length of Participation	2 Years
Number of Study Visits?	6

Additional Information

Phase?	Not Applicable
Study Sponsor?	Rowe, Steven
Study Drugs?	N/A

Eligibility

Age	12 Years and Older
Mutation(s)	Two Copies F508del or One Copy F508del
FEV1% Predicated	No FEV1 Limit
PA Status	N/A
Other	This study is for people with CF who are eligible for the elexacaftor/tezacaftor/ivacaftor triple-combination therapy.

Study Results

STUDY RESULTS NOT YET AVAILABLE

For more information about the results of this study and where it was conducted, visit ClinicalTrials.gov.