

PROSPECT Part A (PROSPECT-OB-14)

Summary

Part A of the PROSPECT study identified biomarkers of CFTR function that could be used to monitor disease progression.

Three groups of participants were enrolled in the study: Group 1: healthy participants that do not have CF, Group 2: individuals with CF who have at least one gene mutation that results in partial CFTR function, and Group 3: individuals with CF who have mutations that result in no CFTR function. Researchers measured lung function and sweat chloride and collected sputum, blood, urine, and stool samples for biomarker measurements. Group 1 participated for two weeks and had two visits. Groups 2 and 3 participated for three months and had three visits.

Specimen Information

Status: Specimens are Available

PROSPECT Part A collected samples from participants at 3 timepoints: Visit 1 (baseline), Visit 2 (14 days after baseline) and Visit 3 (60 days after baseline). Nasal epithelial cells were collected from CF subjects in Groups 2 and 3 at one time point (the samples could be collected at any of the three visits). Group 1 (Healthy Volunteers) only had two visits (Visit 1 and Visit 2) and did not have sputum collected as part of the study.

Visit #	Time From Baseline	Specimens Collected
1	+0 Days	Buffy coat, EDTA plasma, Nasal Epithelial Cells, Serum, Sputum, Urine
2	+14 Days	Buffy coat, EDTA plasma, Serum, Sputum, Stool, Urine
3	+60 Days	Buffy coat, EDTA plasma, Serum, Sputum, Stool, Urine

Study Design

Study Type?	Observational
Randomized Study?	No
Placebo Controlled?	No
Length of Participation	3 Months
Number of Study Visits?	3

Additional Information

Phase?	Not Applicable
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Study Sponsor?**Study Drugs?** N/A**Age** 6 Years and Older**Mutation(s)** No Mutation Requirement**FEV1% Predicated** No FEV1 Limit**PA Status** N/A**Other**

Part A of the PROSPECT study enrolled two groups of people with CF: One group included people with CF that have CF mutations that result in parital CFTR function; the second group included people with CF that have mutations with no CFTR function (specifically those with two copies of F508del).

Study Results

WHAT WE LEARNED:

This study enrolled three groups of participants, Group 1: healthy participants that do not have CF, Group 2: individuals with CF who have a gene mutation that results in partial CFTR function, and Group 3: individuals with CF who have mutations that result in no CFTR function. It found that Group 2 and 3 had similar lung function (FEV1) and that there was a significant difference in sweat chloride concentrations between each group. Additional analyses of the biospecimens collected in the study to identify potential markers of disease severity are ongoing.

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