

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 partial 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](https://clinicaltrials.gov).

PROSPECT Part B (PROSPECT-OB-14 - Part B)

Summary

Part B of the PROSPECT study evaluated the effectiveness of lumacaftor/ivacaftor (Orkambi®) and collected biospecimens and clinical data from people who have two copies of the F508del CFTR mutation both before and after treatment with lumacaftor/ivacaftor.

Participants participated in at least one study visit (baseline visit) prior to being prescribed lumacaftor/ivacaftor. Additional study visits were conducted after participants had taken lumacaftor/ivacaftor for 1, 3, 6 and 12 months. At each visit, researchers measured lung function, body weight and sweat chloride concentration.

Specimen Information

Status: Specimens are Available

PROSPECT Part B collected samples from participants before and the after they began receiving lumacaftor/ivacaftor (Orkambi). Part B was a continuation of Part A and thus the baseline line visit for Part B is "Visit 4". Subsequent visits were timed to occur the number of days indicated after the participant began taking Orkambi. Nasal epithelial cells were collected from CF subjects who consented at one time point (the samples could be collected at any study visit).

Visit #	Time From Baseline	Specimens Collected
4	+0 Days	Buffy coat, EDTA plasma, Nasal Epithelial Cells, Serum, Sputum, Stool, Urine
5	+30 Days	Buffy coat, EDTA plasma, Serum, Sputum, Urine
6	+90 Days	Buffy coat, EDTA plasma, Serum, Sputum, Stool, Urine
7	+6 Months	Buffy coat, EDTA plasma, Serum, Sputum, Urine
8	+12 Months	Buffy coat, EDTA plasma, Serum, Sputum, Urine



Study Design

Study Type?	Observational
Randomized Study?	No
Placebo Controlled?	No
Length of Participation	1 Years
Number of Study Visits?	5

Additional Information

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

Eligibility

Age	12 Years and Older
Mutation(s)	Two Copies F508del
FEV1% Predicated	No FEV1 Limit
PA Status	N/A
Other	Participants in Part B of the PROSPECT study were required to have two copies of the DF508 gene and their physicians planned to treat them with lumacaftor/ivacaftor.

Study Results

WHAT WE LEARNED:

This study found that participants taking lumacaftor/ivacaftor did not have a significant change in lung function (measured by FEV1) but had a significant decrease in sweat chloride and improvements in nutritional status. Additional analyses of the biospecimens collected in the study to identify potential markers of disease progression are ongoing.

DNA was extracted from the PROSPECT study buffy coat samples and genotyped on the Illumina Omni2.5 BeadChip at The Centre for Applied Genomics, The Hospital for Sick Children. Genotype calling was performed using Genome Studio V2011.1. Genotype data is available for request, and should be noted in the sample application.