CF Foundation Specimen Banking

Study Long Title  | G551D Observational Study – Expanded to Additional Genotypes (GOAL-e2)
Short Name        | GOAL-e2 Expansion (Cohorts 2-3, Visits 1-5)
Specimens Currently Available to Other Researchers | Not Yet
Current Study Status | Study Complete

Study Timeline Metrics

<table>
<thead>
<tr>
<th></th>
<th>Planned</th>
<th>Actual (Sponsor)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Begin Enrollment</td>
<td>11/01/2013</td>
<td>02/05/2014</td>
</tr>
<tr>
<td>Complete Enrollment</td>
<td>09/30/2015</td>
<td>12/31/2015</td>
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<tr>
<td>Study Complete</td>
<td>03/31/2016</td>
<td>05/24/2016</td>
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</tbody>
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Number of Patients Planned | 90

General Study Information

Study Type        | Observational
Intervention Type | Control Type
Blinded           | No
Randomized        | No
Duration of Subject Participation | 6 Months
Endpoints Evaluated

Trial Specific Link on ClinicalTrials.gov | http://clinicaltrials.gov/show/NCT01521338
Primary Manuscript Link
Primary Manuscript Citation

Eligibility Criteria

Age       | >= 6 Years
FEV1      | No FEV1 requirement
P. aeruginosa status | Not Applicable
B. cepacia status   | Not Applicable

Other Primary Eligibility Requirements
Must have following CFTR mutations:
For Cohort 2:
a) R117H on at least 1 allele
b) Any known or unknown mutation on the second allele except G551D
For Cohort 3:
b) Any known or unknown mutation on the second allele except G551D OR R117H

Biorepository Specimen Information

General Specimen Information
- WBC, whole blood, serum, and plasma: At every visit, approximately 28 ml of blood will be collected.
- Urine: At every visit, approximately 10 ml will be collected.
- HNE: Collected once during study – not visit specific, from subjects who have consented to HNE Sub-study.
The whole blood (WBCs only and whole blood), serum, plasma, and urine specimens collected will be stored frozen at -70 to -80°C at the site and batch shipped to the CFFT Biorepository for long-term biorepository storage.

Visit # | Time from Baseline | Specimens Collected
--- | ------------------ | -------------------
1   | -30 Months        | serum, EDTA plasma, buffy coat, urine, whole blood
2   | +0 Days           | serum, EDTA plasma, buffy coat, urine, whole blood
3   | +1 Months         | serum, EDTA plasma, buffy coat, urine, whole blood
4   | +3 Months         | serum, EDTA plasma, buffy coat, urine, whole blood
|   | +6 Months | serum, EDTA plasma, buffy coat, urine, whole blood |
