**Study Long Title**  
GOAL - G551D Observational Study (Core)

**Short Name**  
GOAL - G551D Observational Study (Core)

**Specimens Currently Available to Other Researchers**  
Yes

**Current Study Status**  
Closed

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**Study Timeline Metrics**

<table>
<thead>
<tr>
<th>Event</th>
<th>Planned</th>
<th>Actual (Sponsor)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Begin Enrollment</td>
<td>03/01/2012</td>
<td>02/09/2012</td>
</tr>
<tr>
<td>Complete Enrollment</td>
<td>02/28/2013</td>
<td>06/30/2012</td>
</tr>
<tr>
<td>Study Complete</td>
<td>01/10/2013</td>
<td>01/24/2013</td>
</tr>
</tbody>
</table>

**Number of Patients Planned**  
120

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**General Study Information**

**Study Type**  
Observational

**Intervention Type**

**Control Type**

**Blinded**  
No

**Randomized**  
No

**Duration of Subject Participation**  
6 Months

**Endpoints Evaluated**  
Spirometry, Sweat Chloride, Sputum Inflammatory Mediators, Growth, MCC, CFQR, CFRSD, Other PRO

**Trial Specific Link on ClinicalTrials.gov**  

**Primary Manuscript Link**  

**Primary Manuscript Citation**  
Am J Respir Crit Care Med ;DOI 10.1164/rccm.201404-0703OC

**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 the G551D-CFTR mutation on at least 1 allele (any known or unknown mutations allowed in second allele).

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**Biorepository Specimen Information**

**General Specimen Information**

The primary purpose of this study is to collect specimens from patients with the G-551D mutation who may or may not be prescribed Kalydeco. The specimen collection schedule collects one or two baseline samples (before drug) for those prescribed Kalydeco and 3 after drug samples at varying timepoints. For those not prescribed Kalydeco a baseline sample will be collected and a followup sample will be collected once enrollment for the study has closed. The study also evaluated some novel endpoints including mucociliary clearance, sweat rate, pH Pill and sputum inflammatory markers and microbiome in subsets of enrolled subjects.

HNE cells will be collected once during the study (not visit specific), from subjects that have consented to the HNE Sub-study.

**Visit #**  
<table>
<thead>
<tr>
<th>Time from Baseline</th>
<th>Specimens Collected</th>
</tr>
</thead>
</table>
| 1                 | -30 Days  
serum, EDTA plasma, buffy coat, sputum, urine |
| 2                 | +0 Days  
serum, EDTA plasma, buffy coat, urine |
| 3                 | +1 Months  
serum, EDTA plasma, buffy coat, urine |
| 4                 | +3 Months  
serum, EDTA plasma, buffy coat, urine |
| 5                 | +6 Months  
serum, EDTA plasma, buffy coat, sputum, urine |