CF Foundation Specimen Banking	
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

Study Timeline Metrics

	Planned	Actual (Sponsor)
Begin Enrollment	03/01/2012	02/09/2012
Complete Enrollment	02/28/2013	06/30/2012
Study Complete	01/10/2013	01/24/2013

Number of Patients Planned	120

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	http://clinicaltrials.gov/ct2/show/NCT01521338?term=goal+and+cystic+fibrosis&rank=1
Primary Manuscript Link	http://www.atsjournals.org/doi/abs/10.1164/rccm.201404-0703OC?url_ver=Z39.88-2003&rfr_id=ori:rid:crossref.org&rfr_dat=cr_pub%3dpubmed&#.U63OQZRdWaU
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).

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 mucocilliary 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 #	Time from Baseline	Specimens Collected
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