

CF Foundation Specimen Banking			
<b>Study Long Title</b>	G551D Observational Study – Expanded to Additional Genotypes (GOAL-e2)		
<b>Short Name</b>	GOAL-e2 Expansion (Cohorts 2-3, Visits 1-5)		
<b>Specimens Currently Available to Other Researchers</b>	Not Yet		
<b>Current Study Status</b>	Study Complete		
Study Timeline Metrics			
	Planned	Actual (Sponsor)	
Begin Enrollment	11/01/2013	02/05/2014	
Complete Enrollment	09/30/2015	12/31/2015	
Study Complete	03/31/2016	05/24/2016	
<b>Number of Patients Planned</b>	90		
General Study Information			
<b>Study Type</b>	Observational		
<b>Intervention Type</b>			
<b>Control Type</b>			
<b>Blinded</b>	No		
<b>Randomized</b>	No		
<b>Duration of Subject Participation</b>	6 Months		
<b>Endpoints Evaluated</b>			
<b>Trial Specific Link on ClinicalTrials.gov</b>	<a href="http://clinicaltrials.gov/show/NCT01521338">http://clinicaltrials.gov/show/NCT01521338</a>		
<b>Primary Manuscript Link</b>			
<b>Primary Manuscript Citation</b>			
Eligibility Criteria			
<b>Age</b>	>= 6 Years		
<b>FEV1</b>	No FEV1 requirement		
<b>P. aeruginosa status</b>	Not Applicable		
<b>B. cepacia status</b>	Not Applicable		
<b>Other Primary Eligibility Requirements</b>	<p>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:  a) A Non-G551D gating mutation on one allele: (G178R, S549N, S549R, G551S, G970R, G1244E, S1251N, S1255P, G1349D)  b) Any known or unknown mutation on the second allele except G551D ORR117H  Cannot be participating in the VX-770-105, VX-770-106, VX-770-108, VX-770-110, VX-770-111, VX-770-112, or VX-770-113 study, VX-770 Extended Access Program or use of ivacaftor within 6 months prior to Visit 1.</p>		
Biorepository Specimen Information			
<b>General Specimen Information</b>	<ul style="list-style-type: none"> <li>• WBC, whole blood, serum, and plasma: At every visit, approximately 28 ml of blood will be collected.</li> <li>• Urine: At every visit, approximately 10 mL will be collected.</li> <li>• HNE: Collected once during study – not visit specific, from subjects who have consented to HNE Sub-study.</li> </ul> <p>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.</p>		
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	

5	+6 Months	serum, EDTA plasma, buffy coat, urine, whole blood
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