



CLINICAL
TRIALS
TRANSFORMATION
INITIATIVE

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Using the Cystic Fibrosis Patient Registry to Support Clinical Trials Planning and Set-up

Aliza Fink, DSc

Cystic Fibrosis Foundation

Disclaimer

- The views and opinions expressed in this presentation are those of the individual presenter and do not necessarily reflect the views of the Clinical Trials Transformation Initiative.
- The presenter is an employee of the Cystic Fibrosis Foundation. To advance drug development and a search for a cure, Cystic Fibrosis Foundation has contractual agreements with several companies to receive royalties related to drugs that are developed as a result of CFF funding. Any royalties we receive are used in support of our mission.

Cystic Fibrosis

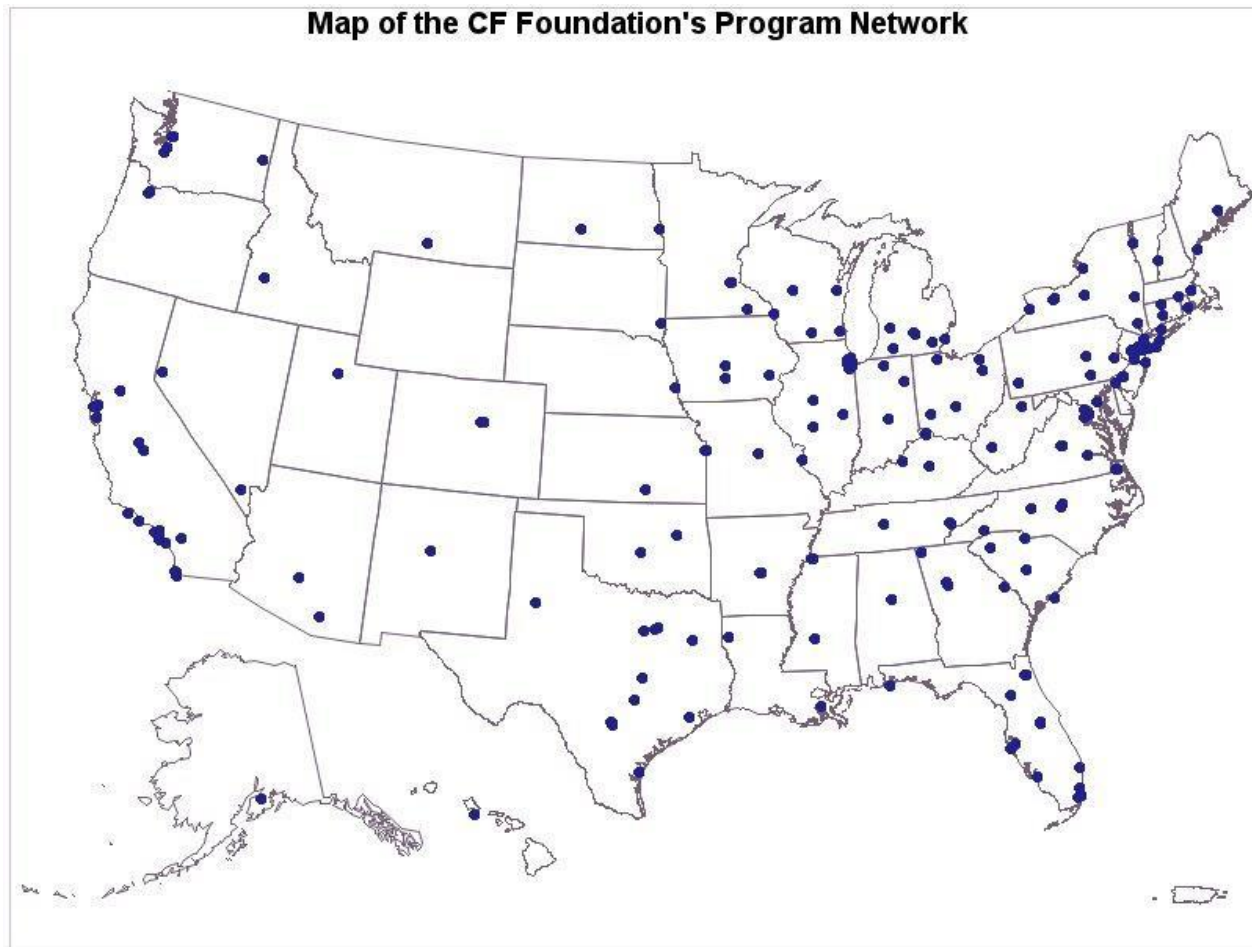
- Rare Disease: about 35,000 people in the US
- Caused by mutations in the CFTR gene

Prevalence	# of mutations
5+%	1
1 - <5%	10
<1%	~2000

- Diagnosed early in life: universal newborn screening since 2010
- Current life expectancy at birth ~44 years
- Treatment regimen: 2 – 3 hours per day

CF Foundation Care Network

- CF Foundation accredits care centers throughout the United States
- Care Centers are comprised of: pediatric, adult and affiliate care programs
- Participation in the registry is an requirement of accreditation



Data Collected in the CFFPR

Inclusion criteria

- Clinical diagnosis of CF
- Seen at care center
- Consent to participate

Data collected at:

- Diagnosis
- Clinic Visits
- Hospitalizations / Home
- IV treatments
- Annually

WHAT TYPES OF INFORMATION CAN YOU FIND IN THE CYSTIC FIBROSIS FOUNDATION PATIENT REGISTRY?



DIAGNOSIS

- Age at diagnosis
- Method of diagnosis: newborn screening, respiratory and/or gastrointestinal symptoms, failure to thrive
- CFTR gene mutations
- Sweat test results



CARE RECEIVED

- Location of care: clinic, hospital or home
- Providers seen during clinic visit
- Reason for hospitalization: pulmonary exacerbation, transplant, gastrointestinal
- Length of hospital stay



DEMOGRAPHICS

- Age
- Sex
- Race
- Ethnicity
- Vital status
- State of residence
- Personal and parental education
- Employment status



- Marital status
- Smoking status
- Health insurance coverage

TREATMENTS

- Antibiotics
- Mucus thinners
- Bronchodilators
- Anti-inflammatories
- Airway clearance techniques
- Pancreatic enzymes
- Nutritional supplements



- CFTR modulators
- Growth hormone
- Insulin
- Oxygen

OTHER CONDITIONS AND EVENTS

- CF-related diabetes
- Asthma
- Sinus disease
- Gastroesophageal (acid) reflux disease (GERD)
- Liver disease
- Allergic bronchial pulmonary aspergillosis (ABPA)
- Osteoporosis
- Depression and anxiety
- Pregnancy



- Transplant: lung, liver, kidney

MEASUREMENTS & SCREENING TESTS

- Height and weight
- Lung function
- Cultures: *Pseudomonas aeruginosa*, *Staphylococcus aureus*, *Burkholderia cepacia* complex, nontuberculous mycobacteria
- Pancreatic function
- Screenings: mental health, bone health, CF-related diabetes
- Blood tests: glucose, liver & kidney function, vitamin levels



See the Annual Data Report for a full list of the data collected by the CF Foundation Patient Registry: www.cff.org/Research/Researcher-Resources/Patient-Registry/CF-Patient-Registry-Reports

Current Status of the Registry

PATIENT REGISTRY BY THE NUMBERS



282

CF care programs



29,887

People in the Registry



129,542

Clinic visits



22,535

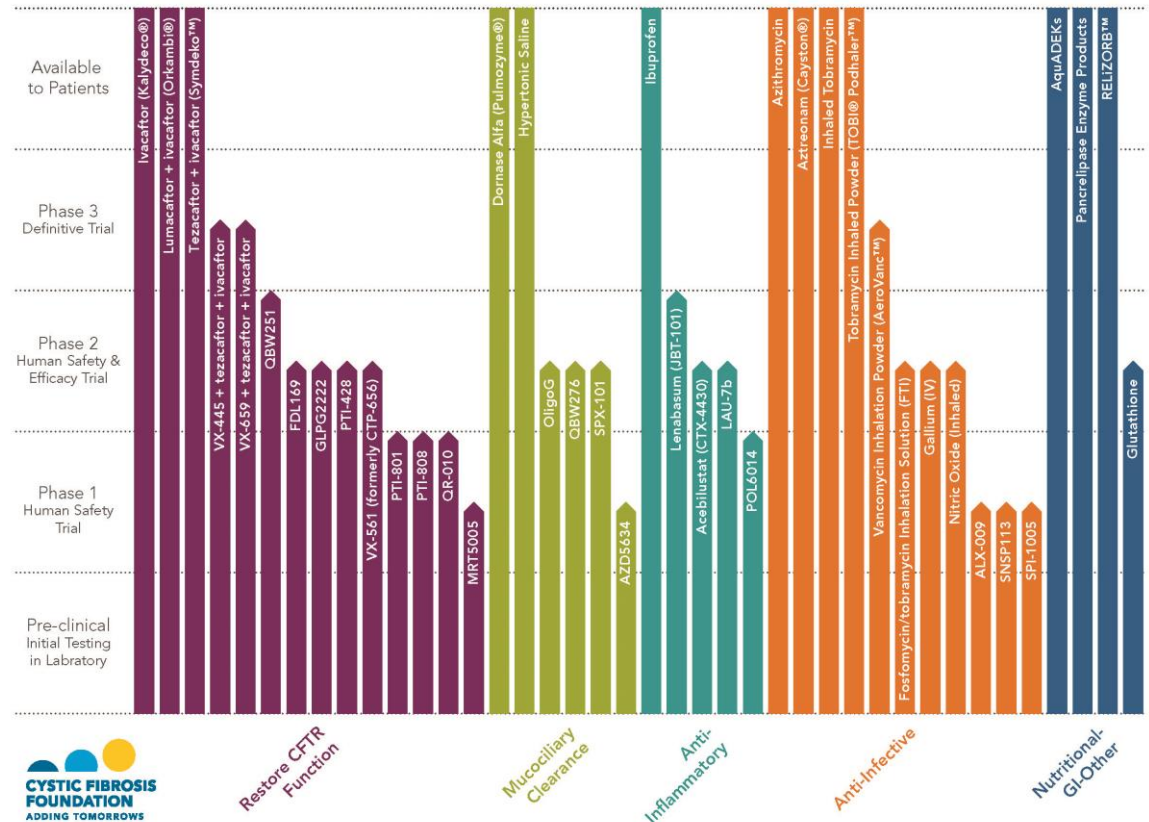
Hospitalizations

Therapeutics Development Network

- Subset of the CF Foundation Care Network
- Conduct industry and non-industry funded studies
- Currently, about 30 studies in progress

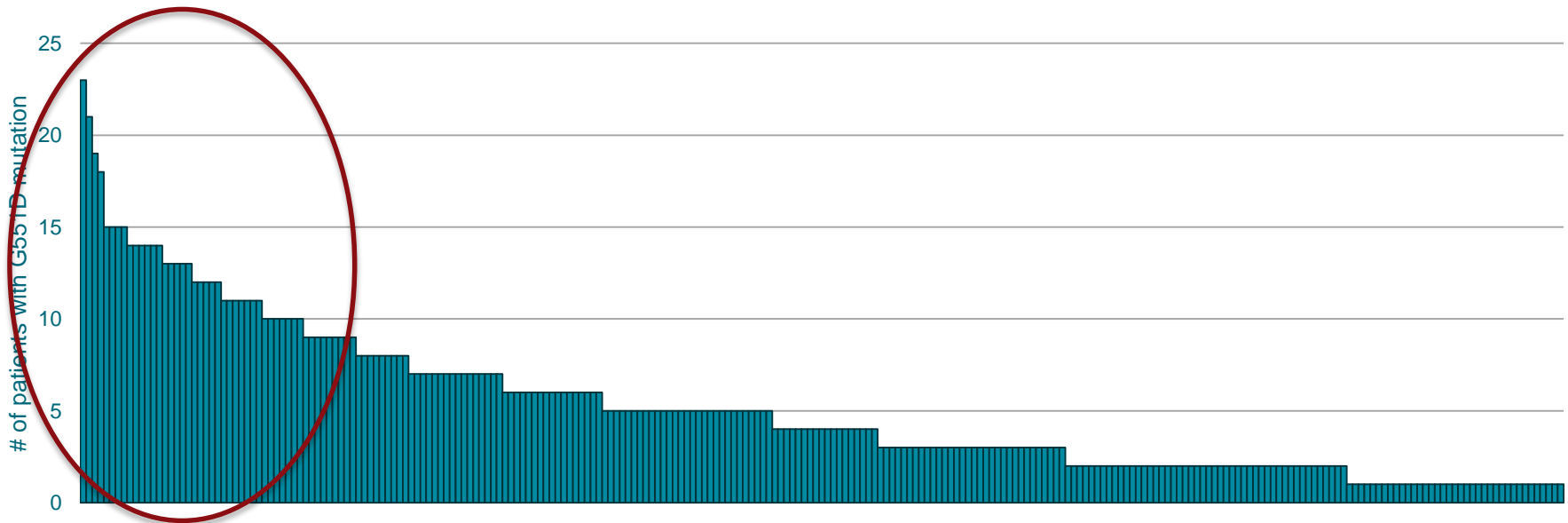
Drug Development Pipeline

4/18/2018



Site Selection

- CFTR modulators – treat underlying disease, indication is based on CFTR mutation
- First CFTR modulator needed to be tested among people with the G551D mutation, found in ~4% of the population.



Clinical Trials in CFSmartReports

CYSTIC FIBROSIS FOUNDATION CFSmartReports

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Please choose your center: * 9001/9001:My Test Pediatric Program

Solicitations Recruiting Studies Referral Studies


Trial Name	Sponsor	Study Phase	Status	Study Type	Intervention Type	Potentially eligible Patients
Phase 2			Protocol Reviewed	Observational		Potentially eligible Patients
(CX-)			Protocol Reviewed	Interventional	Nutritional-GI Therapies	Potentially eligible Patients
Laurent Phase 2 APPLAUD		2	Protocol Reviewed	Interventional	Anti-Inflammatory	Potentially eligible Patients
Novartis QBW276 Cohorts 1 and 2		2	Protocol Reviewed	Interventional	Mucociliary Clearance	Potentially eligible Patients
Novartis QBW276 Cohort 3		2	Protocol Reviewed	Interventional	Mucociliary Clearance	Potentially eligible Patients

Studies that are soliciting programs to participate


Studies that have selected programs and are enrolling participants

Studies that are referring patients to enrolling programs

List of Potentially Eligible Patients



CFSmartReports


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Study: VX-659-103 DF508 Homozygous
Sponsor: Vertex **Study Phase:** 3 **Status:** Protocol Reviewed
Study Type: Interventional **Intervention Type:** Restore CFTR Function **Date query deployed:** 2/22/2018
Program Number : 9002

Number of Potentially Eligible Patients: 8

List can be exported to Excel 

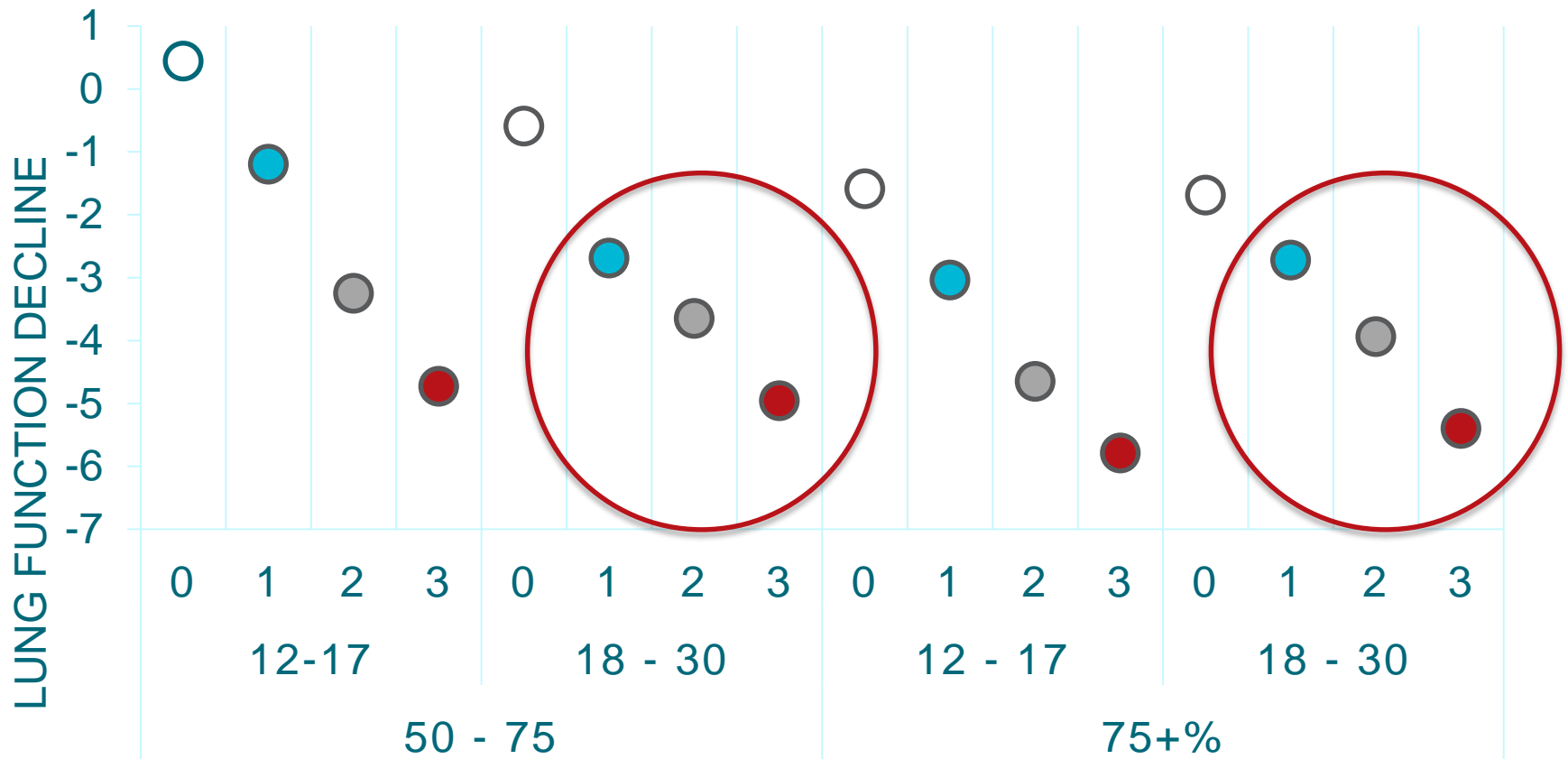
	Name	Reviewed	CFF ID	Current Age	Mutation 1	Mutation 2	Last FEV1 % Predicted	Cirri
Edit	Mugwort, Halueve	Not Reviewed ▼	9928936	14	F508del	F508del	52.4	
Edit	Thejor, Lusha	Not Reviewed ▼	9900004	25	F508del	F508del	51.1	
Edit	Sylmaris, Sharian	Not Reviewed ▼	9928923	32	F508del	F508del		05/
Edit	Paceran, Skongririka	Not Reviewed ▼	9900043	45	F508del	F508del	41.9	
Edit	Phipetor, Taylor	Not Reviewed ▼	9900025	39	F508del	F508del	65.4	
Edit	Hopesinger, Ygrainne	Not Reviewed ▼	9900010	28	F508del	F508del	63.0	
Edit	Chaosarmour, Ghilanna	Not Reviewed ▼	9900021	14	F508del	F508del	72.9	
Edit	Presberos, Ceufroy	Not Reviewed ▼	9928913	29	F508del	F508del	85.1	02/

Clicking the patient's name shows a report with their clinical data over the past 2 years.

Developing Inclusion Criteria

➤ Outcome of interest – lung function decline

➤ Which patients have the highest risk of outcome?



Increasing study efficiency

- ▶ FDA mandated study of rare adverse event among individuals taking pancreatic enzyme replacement therapy.

10 Year Surveillance for Suspected Fibrosing Colonopathy

2-year enrollment

8 years of additional surveillance

Base Population:

- Patients enrolled in CFF Patient Registry with CF diagnosis;
- Treated at IRB approved site; and
- Had a regular clinic visit after IRB approval AND before the end of the two year enrollment period

Numerator Population:

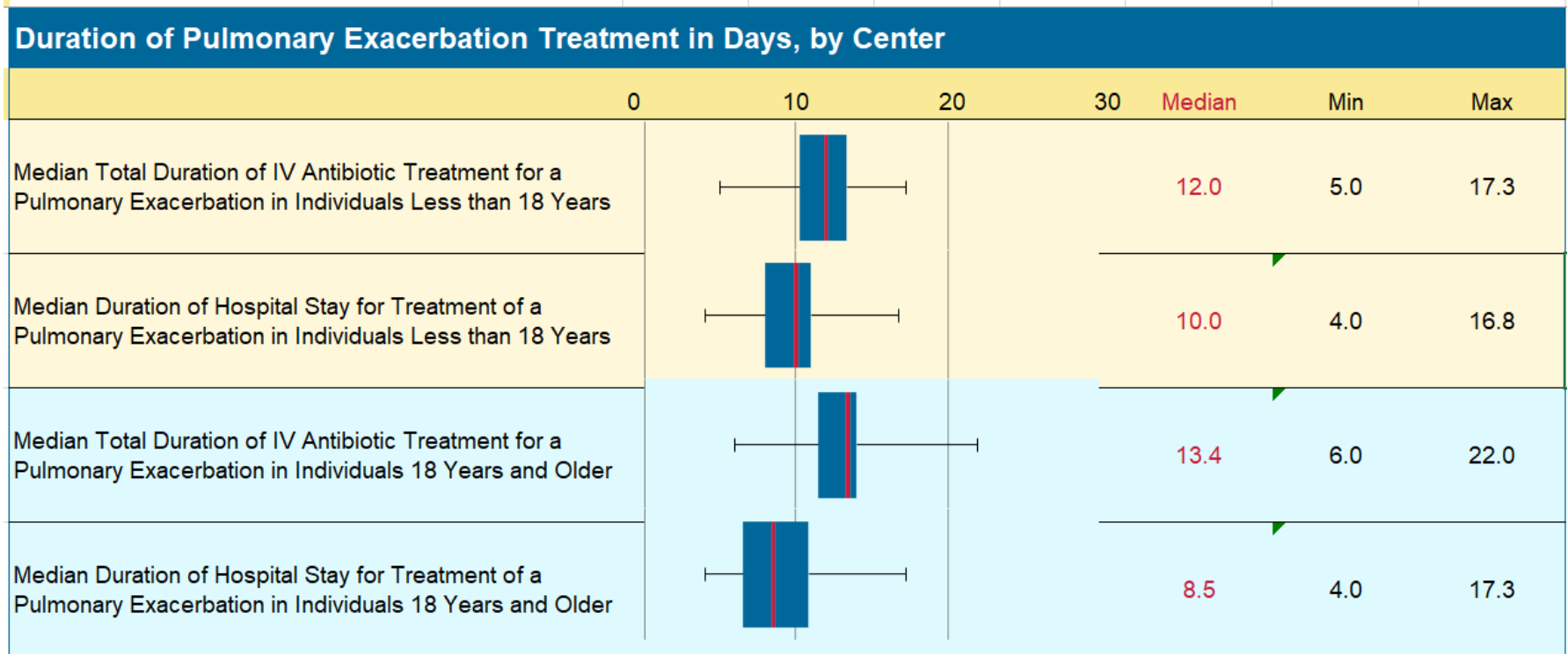
- Patients that meet the criteria for the base population;
- Patients that meet the inclusion/exclusion criteria for suspected fibrosing colonopathy;
- Patient provided study specific informed consent to release medical information for independent, blinded adjudication committee

**PERT Exposure Data Supplied from
CFF Patient Registry**

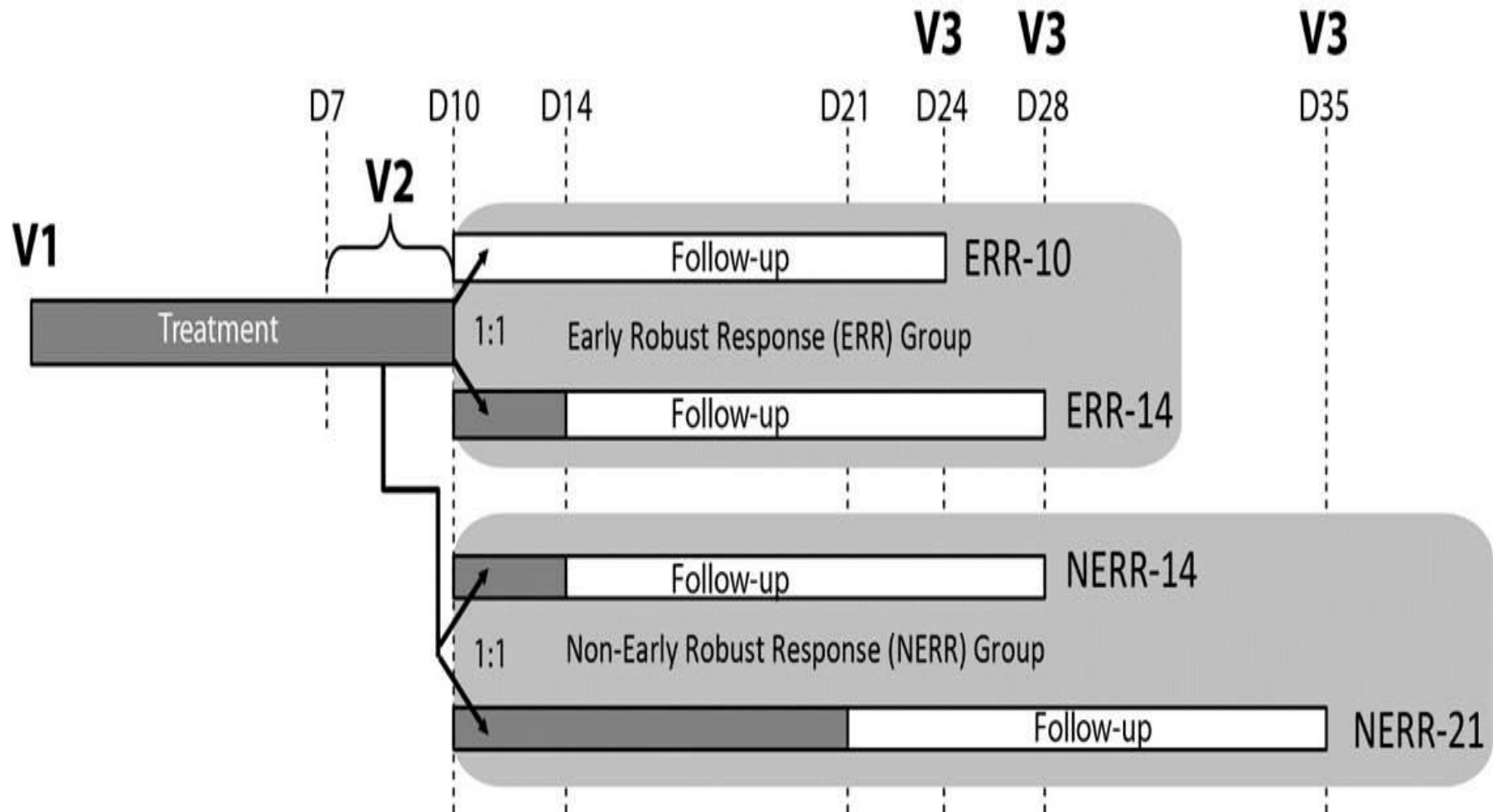


**Numerator Case Data From Separate
Case Report Form NOT Present in CFF
Registry, but Needed for Adjudication**

From research question...



.... To implementation



Strengths and Limitations

Strengths

- Comprehensive (people and data)
- Contains clinical data
- Longitudinal

Limitations

- Timeliness
- Difference in variable definitions
- Not generalizable to other diseases

THANK YOU.



Aliza Fink

afink@cff.org



www.ctti-clinicaltrials.org