CLINICAL TRIALS TRANSFORMATION INITIATIVE

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

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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

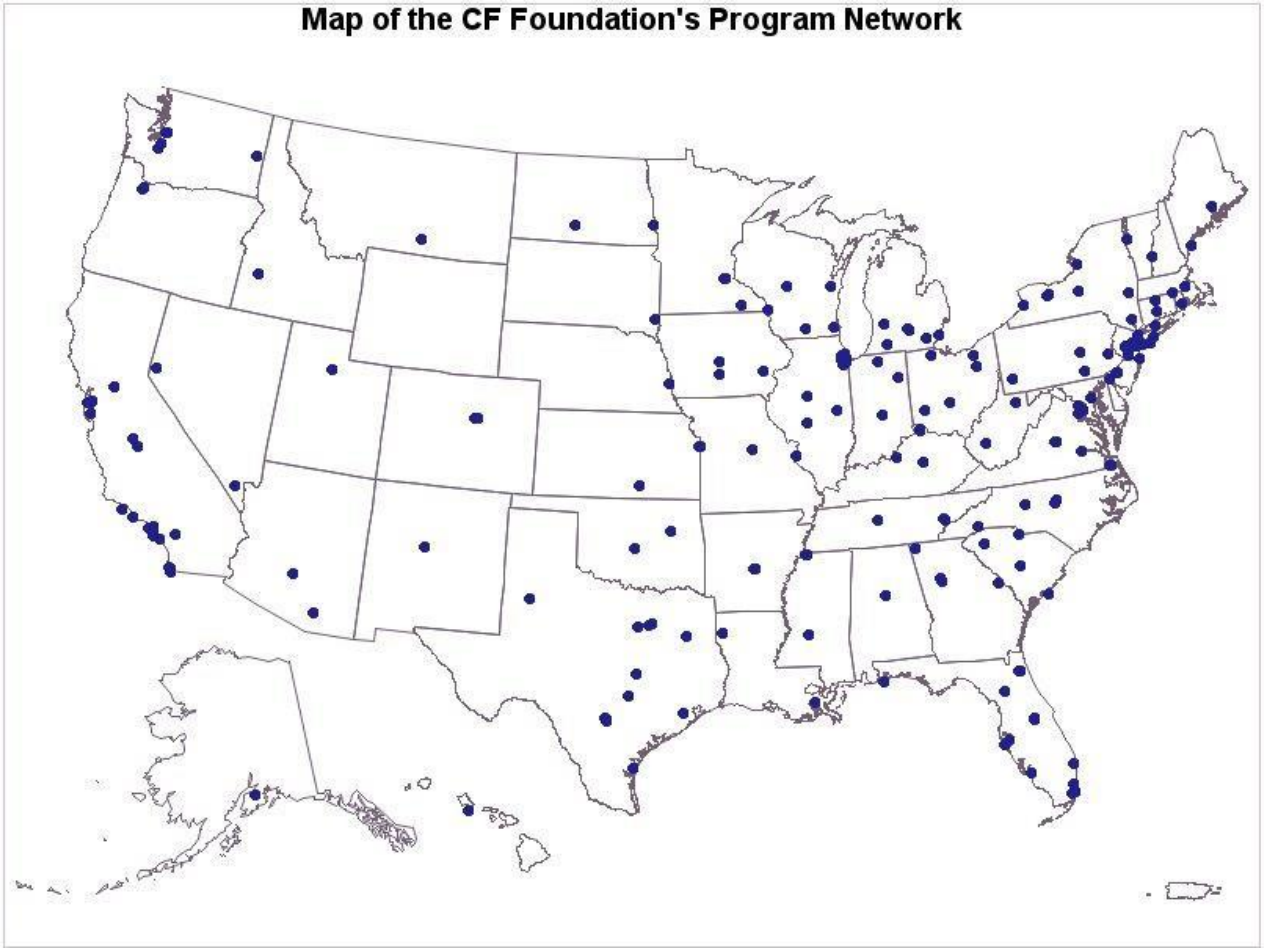
- Rare Disease: about 35,000 people in the US
- Caused by mutations in the CFTR gene

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



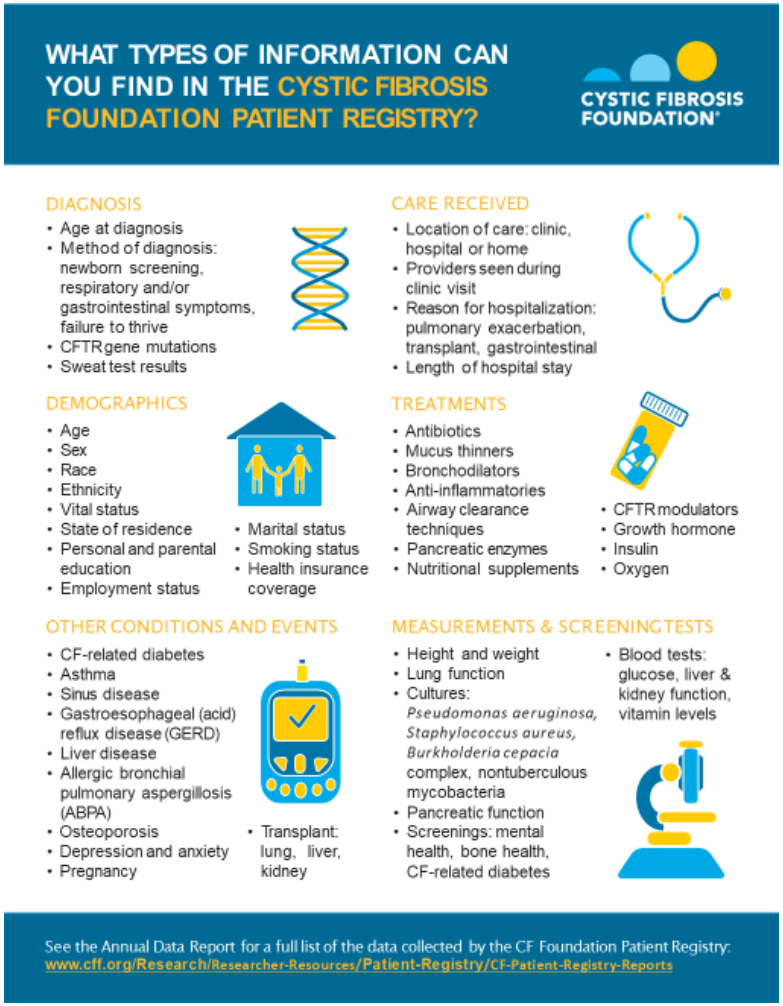
Data Collected in the CFFPR

Inclusion criteria

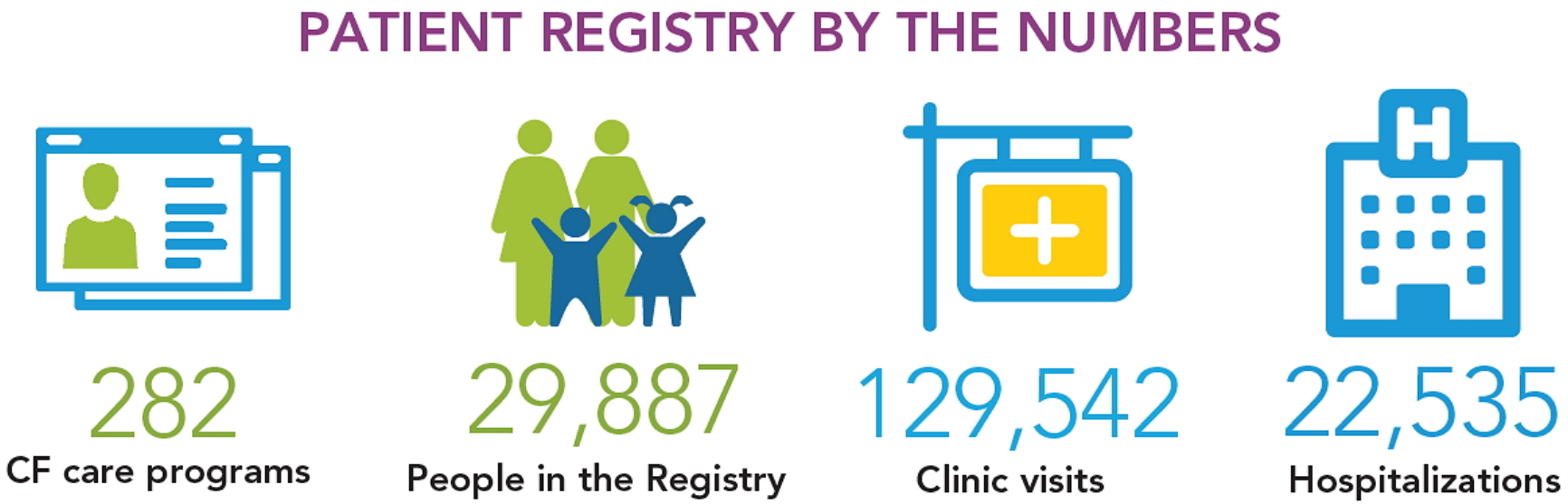
- Clinical diagnosis of CF
- Seen at care center
- Consent to participate

Data collected at:

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

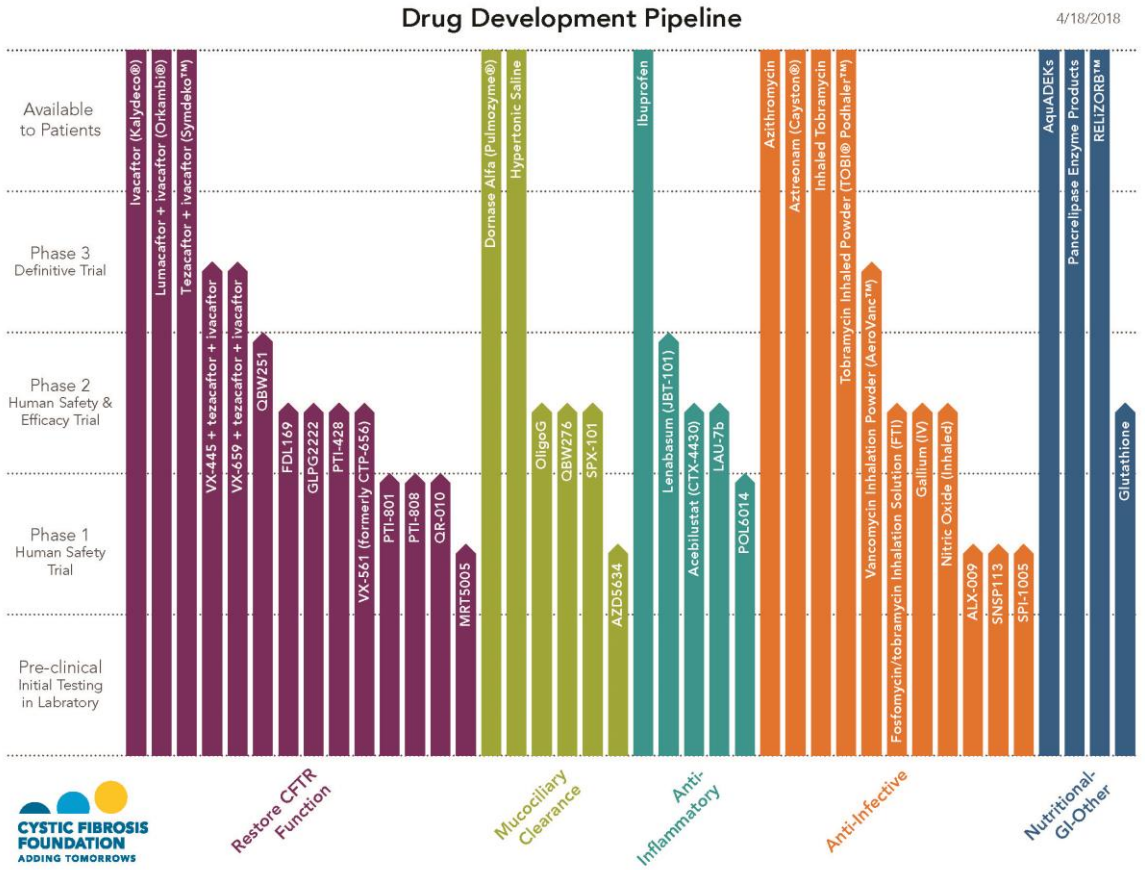


Current Status of the Registry



Therapeutics Development Network

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



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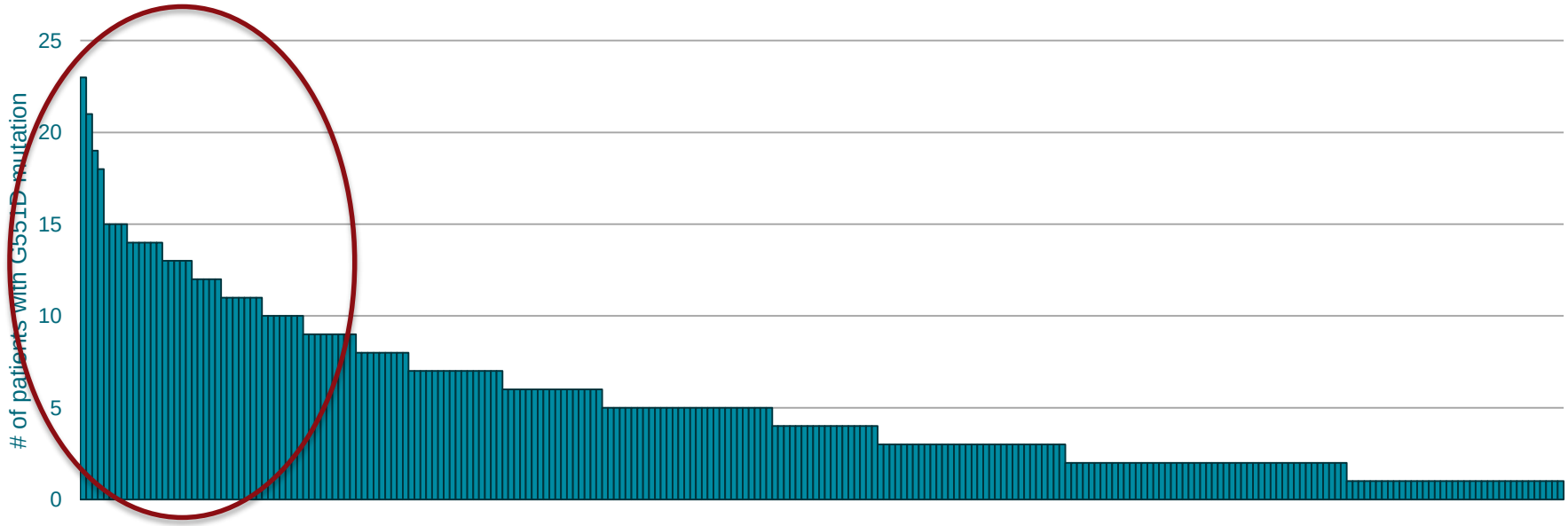
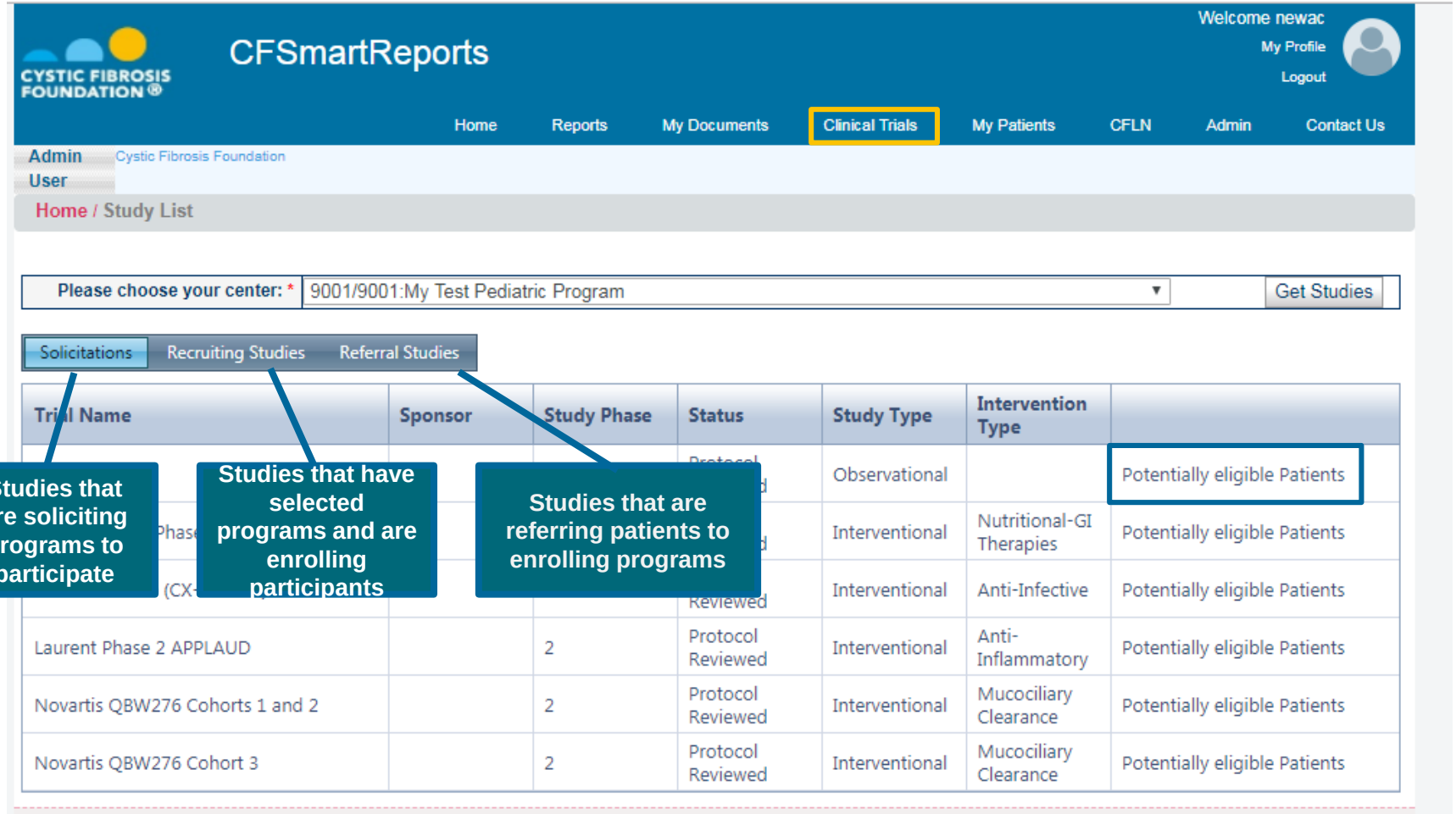
Site Selection

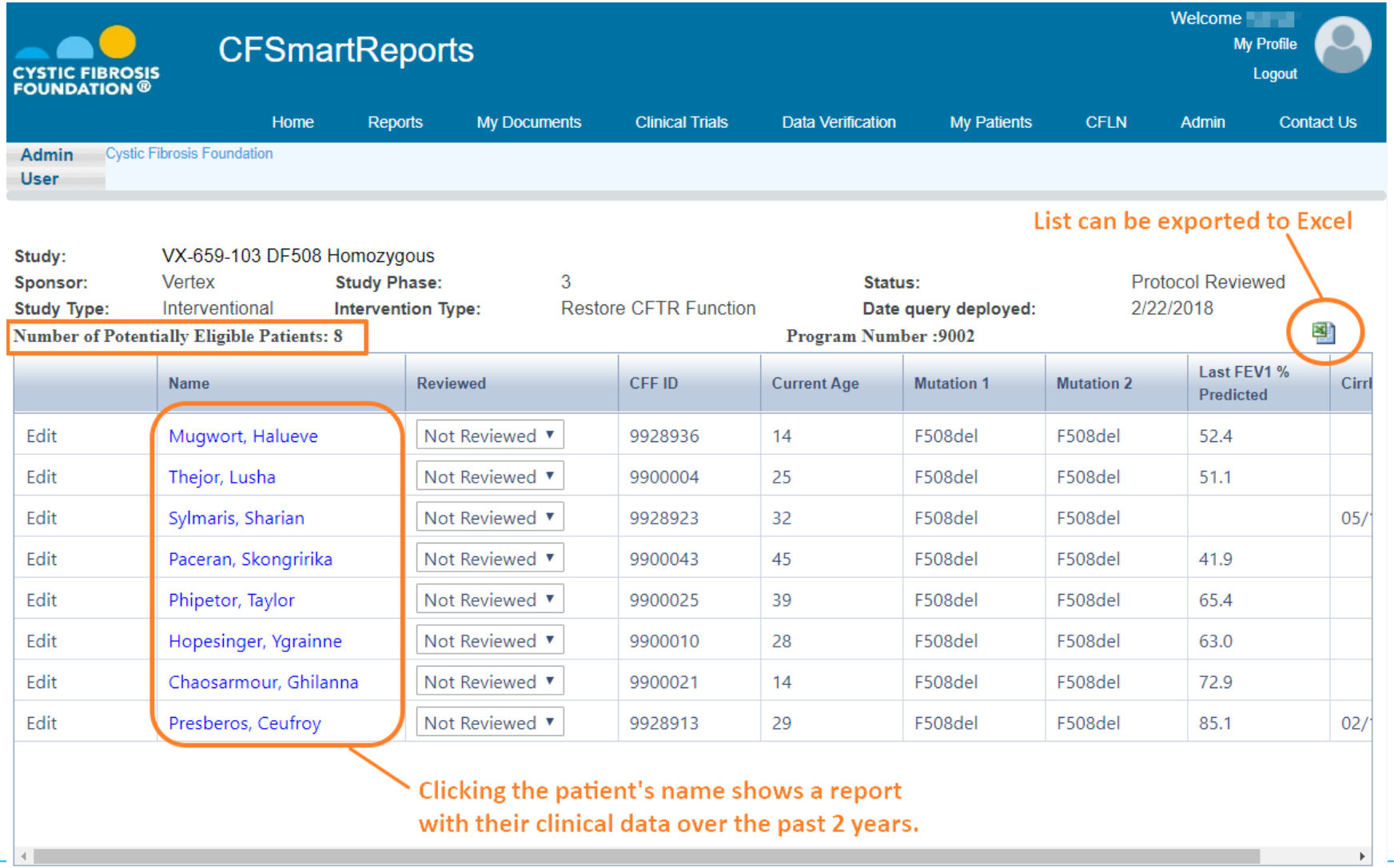
- CFTR modulators – treat underlying disease, indication is based on CFTR mutation
- First CFTR modulator needed to be tested among people with the G551D mutation, found in ~4% of the population.



Clinical Trials in CFSmartReports

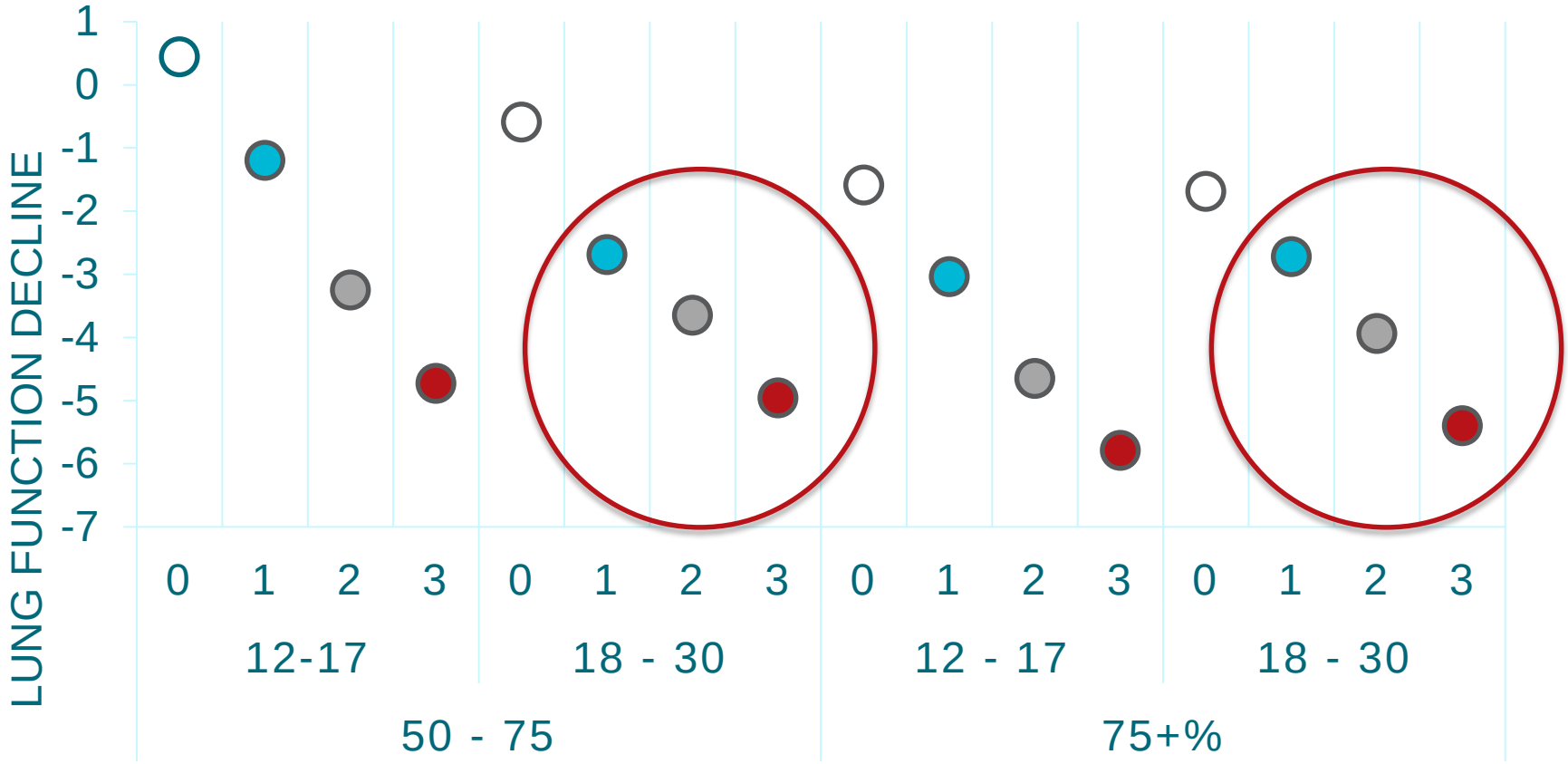


List of Potentially Eligible Patients



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

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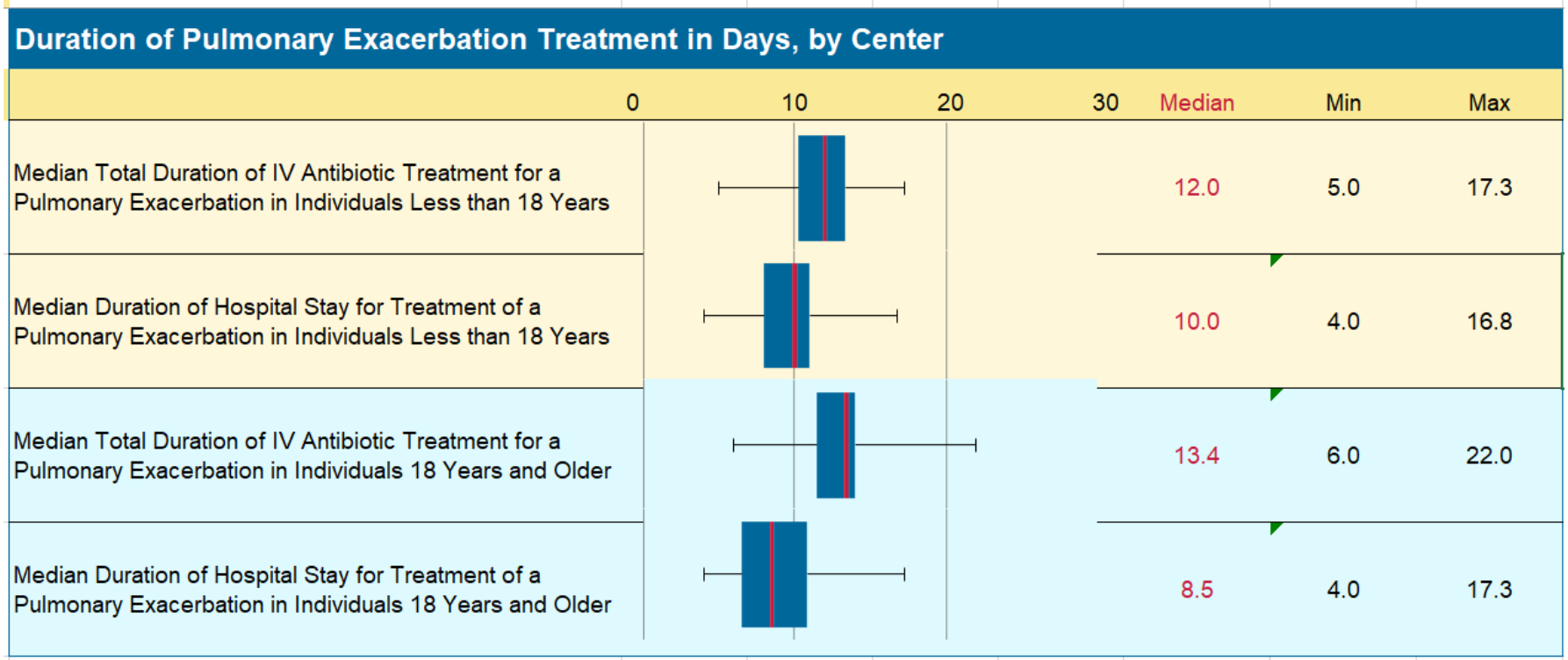
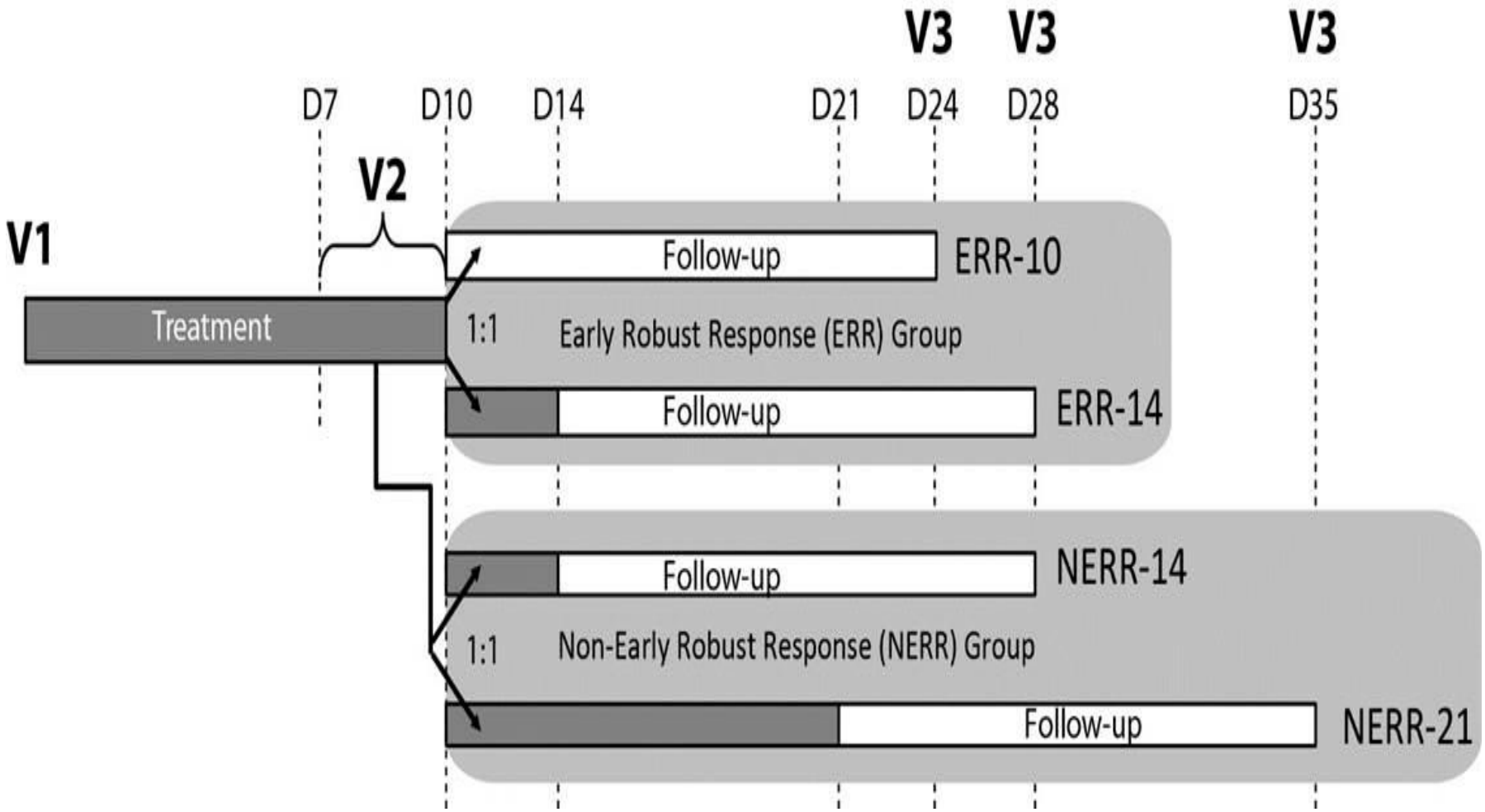
From research question…



…. 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.

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