The Cystic Fibrosis Foundation Experience

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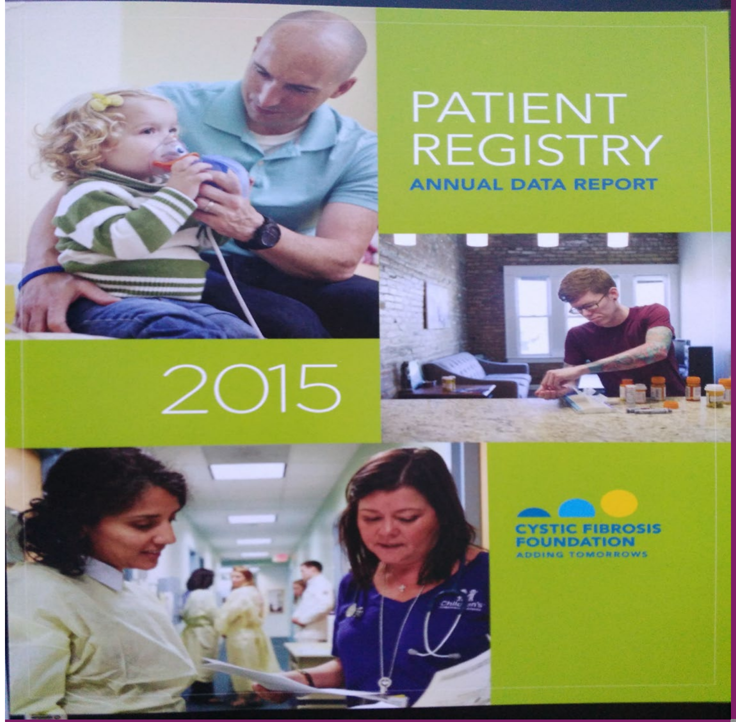
Cornerstones Set in Place



Patient Registry

Benefits:

- Natural history of the disease
- Track the impact of interventions

CF Care Center Network

Benefits:

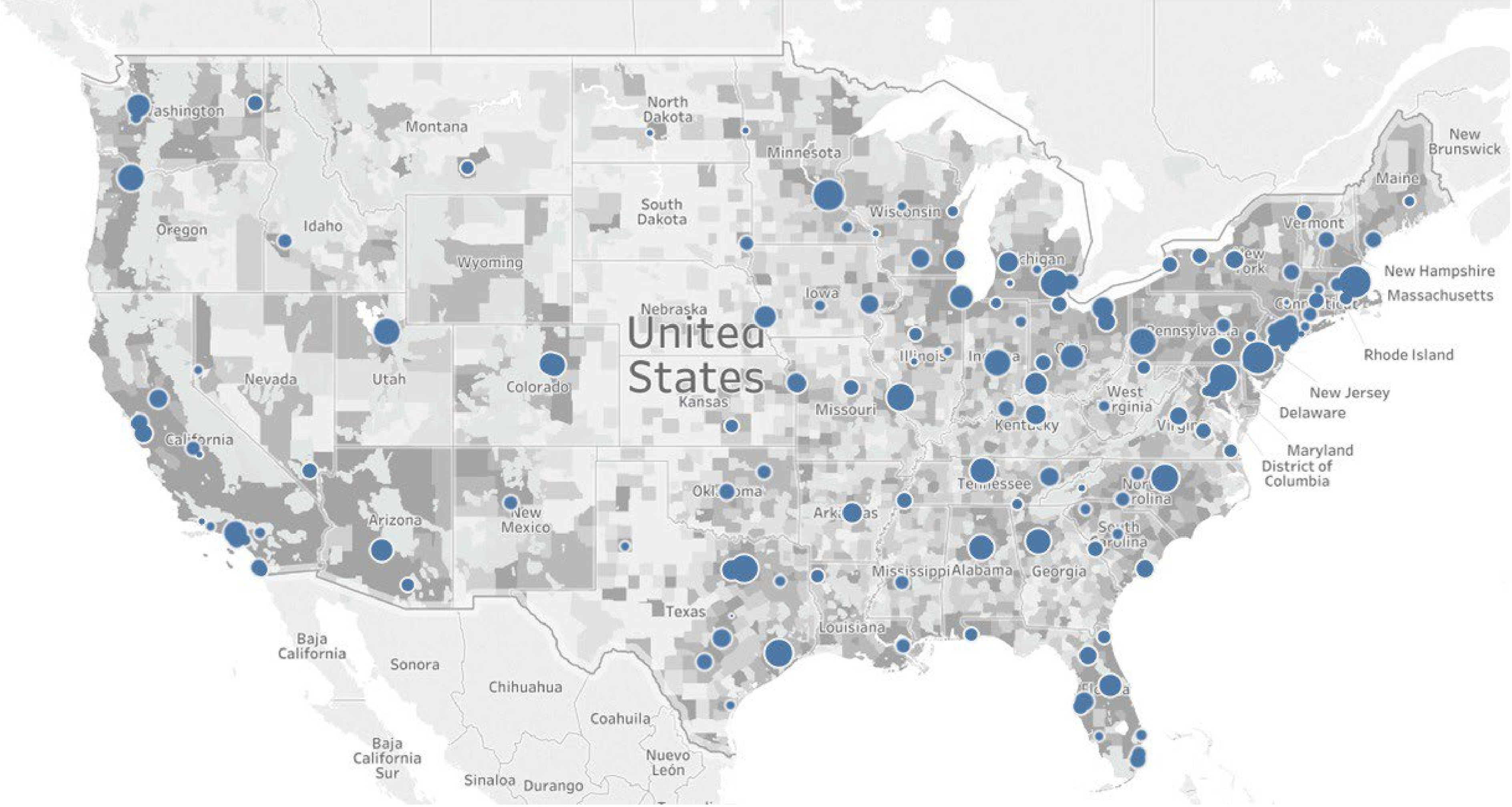
- Interdisciplinary care
- Descriptive research
- Exposure to faculty and trainees

CF Foundation Care Center Network

Pediatric Programs 132

Adult Programs 119

Affiliate Programs 35



High-Quality Specialized Care: Patient Registry

PATIENT REGISTRY
2021
ANNUAL DATA REPORT

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From 1986 through the end of 2014:

- 48,463 unique patients
- 632,022 person-years of data,
- 2,497,178 clinic visits, and
- 241,984 hospitalizations and/or home IV episodes

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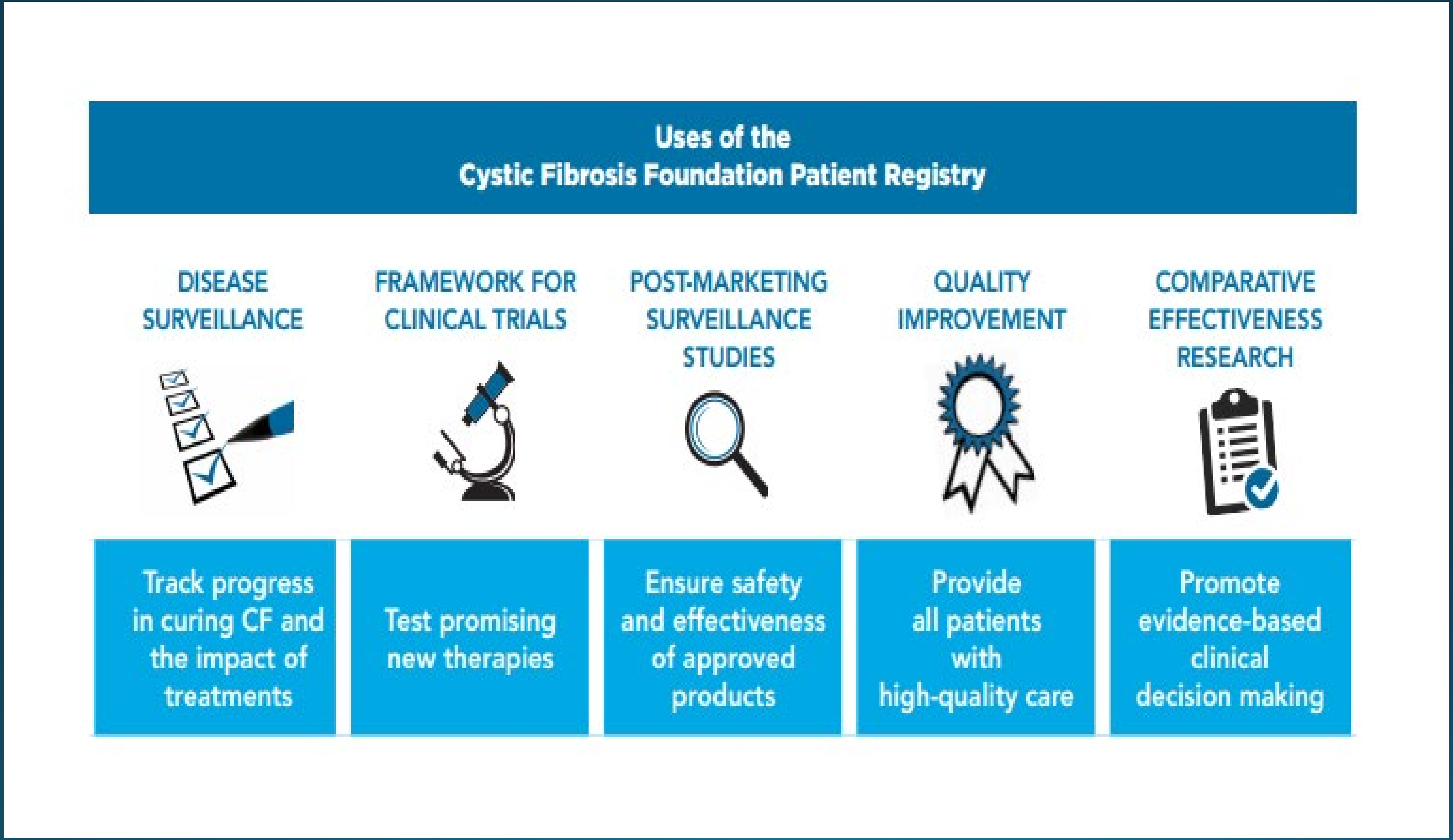
ADDING TOMORROWS

Knapp EA, et al. Ann Am Thorac Soc 2016;13(7):1173-1179



Uses of the Cystic Fibrosis Foundation Patient Registry

DISEASE SURVEILLANCE	FRAMEWORK FOR CLINICAL TRIALS	POST-MARKETING SURVEILLANCE STUDIES	QUALITY IMPROVEMENT	COMPARATIVE EFFECTIVENESS RESEARCH
Track progress in curing CF and the impact of treatments	Test promising new therapies	Ensure safety and effectiveness of approved products	Provide all patients with high-quality care	Promote evidence-based clinical decision making

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CF Lung Transplant Consortium (CFLTC)
How did we get here?

- During the last Comprehensive Strategic Planning Initiative in 2012- Lung Transplant was identified by the CF community as an area that was not currently being addressed by the Foundation

- The CF Foundation hosted the first CF Lung Transplantation Summit in early 2016

- Spring 2016, the CF Foundation announced a multi-year $15 million investment (invested close to $70 million over last 7 years)

Establish a collaborative network of clinical transplant centers

Resources for our patients and families as well as CF providers and transplant programs

Empower people with CF by supporting and guiding them as they pursue the transplant journey

Advocate for affordable and accessible healthcare for our patients and families

Fund basic science research

Areas of Need Endorsed by Strategic Planning Steering Committee

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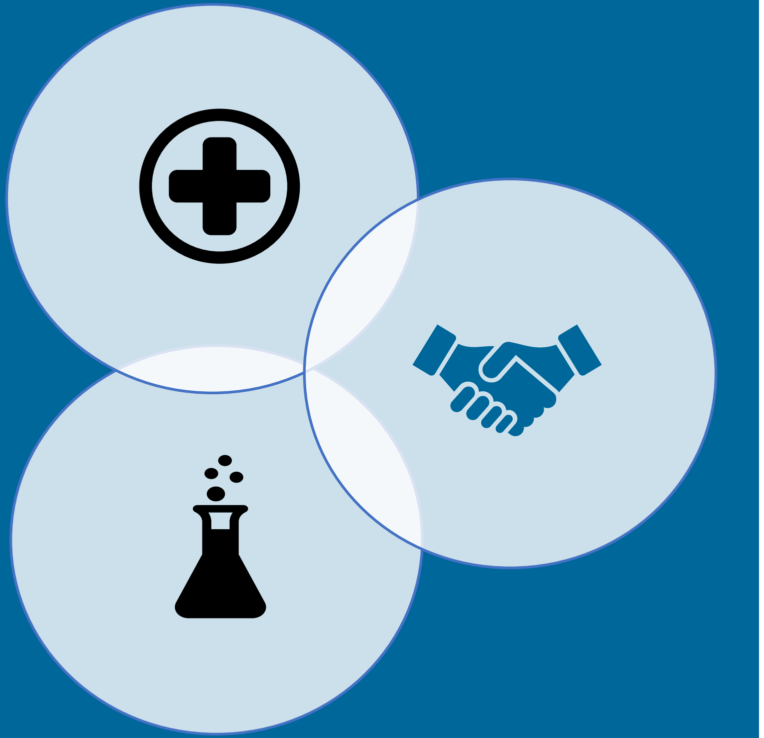
Cystic Fibrosis Lung Transplant Consortium Established in 2017

Purpose and Objectives

- To establish a group of lung transplant centers committed to improving outcomes of lung transplant through quality improvement, guidelines development, and research focused on Chronic Lung Allograft Dysfunction (CLAD).
- To promote relationships between Cystic Fibrosis (CF) and lung transplant care teams to enhance the care of individuals with CF and advanced lung disease.
- To provide a forum for the exchange of ideas about the science and art of lung transplantation.
- To establish and maintain a prospective central repository of biospecimens from lung transplant recipients ("the CFLTC Biorepository") and a clinical database (the "CFLTC Registry") for clinical and translational research on CLAD.



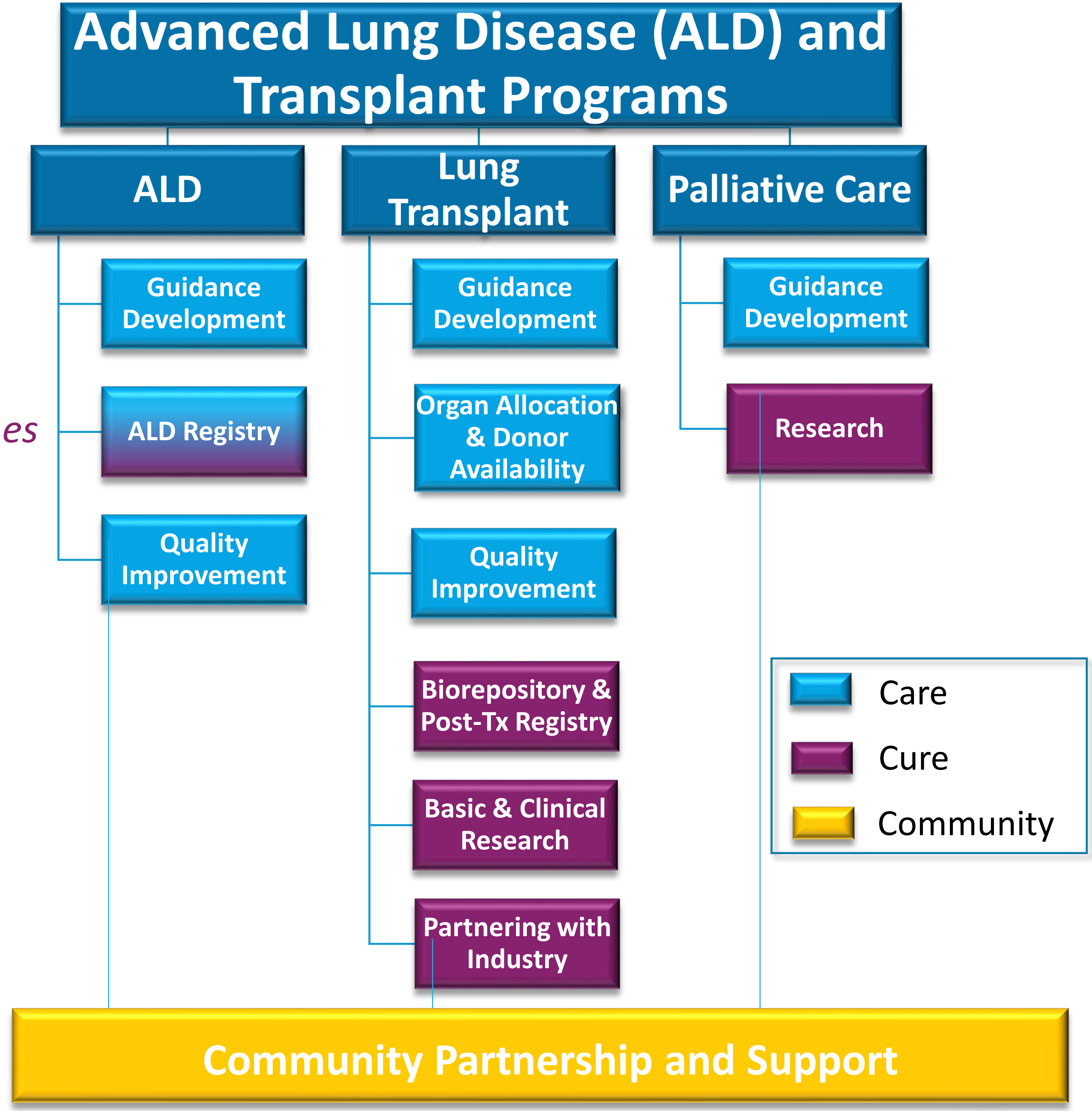
Cystic Fibrosis Lung Transplant Consortium (CFTLC)

10 CFLTC Centers selected in 2017 via competitive application process:

- The Cleveland Clinic
- Duke University
- Johns Hopkins University
- Loyola University
- University of Minnesota
- University of Pennsylvania
- University of Pittsburgh
- University of Toronto
- University of Washington Seattle
- Washington University in St. Louis

5 Centers added in 2020 through a competitive application process:

- Columbia University
- Massachusetts General
- Stanford University
- University of California, Los Angeles
- University of California, San Francisco



Advanced Lung Disease (ALD) and Transplant Programs

ALD
- Guidance Development
- ALD Registry
- Quality Improvement

Lung Transplant
- Guidance Development
- Organ Allocation & Donor Availability
- Quality Improvement
- Biorepository & Post-Tx Registry
- Basic & Clinical Research
- Partnering with Industry

Palliative Care
- Guidance Development
- Research

Community Partnership and Support

🟦 Care

🟪 Cure

🟨 Community

Mission:

To improve the care and long-term outcomes of individuals with CF and ALD by optimizing decision-making and access to lung transplantation, and improving outcomes after lung transplantation

CFF ALD & Lung Transplant Highlights

Multifaceted approach to improve the care and outcomes of individuals with ACFLD including their transplant journeys

Care

- Diversity, Equity, and Inclusion to be included in all aspects of work
- Continued collection and analysis of ALD data from the Patient Registry
  - The effect of Trikafta long-term
- Post-transplant shared care
- Educational Resources

Community

- Continued partnership of Community Members on steering committees, guideline developments, and review of research proposals
- Small Group Discussions

Research

- CLAD and post- lung transplant research:
  - CFLTC Registry & Biorepository
  - 2024 CLAD & CTOT RFAs
  - CLAD Working Group
  - FDA Collaboration

Encouraging Research Proposals

- CFLTC centers or non-consortium investigators who wish to collaborate with consortium sites on a research project may submit a project description by following protocols established and implemented by the CFF

- The Officers will review the project description, evaluate its feasibility and alignment with the CFLTC mission, and determine if the project will be brought to the Steering Committee for consideration.

- A 2/3 majority vote of the Steering Committee present and voting will be required for adoption of the research proposal. A quorum of 2/3 of committee members is required for voting.

- In addition to CFLTC research proposals, competitive RFAs will be published as needed for lung transplant related funding opportunities for consortium and non-consortium applicants.

Highlights – CFF Research Funding

2016 – Improving CF LT Outcomes RFA

Mechanism(s) of LT rejection, approaches to improve survival

2018 – Advancing Research Solutions to CLAD RFA

Mechanisms involved in CLAD, therapies to prevent/treat CLAD

2021 – Approaches to early diagnosis & treatment of CLAD RFA

Biomarkers of CLAD → detection, prevention and/or treatment targets

2022 – CTOT ancillary studies RFA

Access to well-characterized CTOT specimens/clinical data to study CLAD

2023 – CFLTC ancillary study of Airway Brushes

Bronchial brush & BAL collection linked to CFLTC Registry

CFLTC Biorepository and Transplant Registry Background

CFLTC Convened 2016

Care and Quality Improvement Focus with long-term vision of collaborative research infrastructure

Coordinating Center RFP 2019

Biorepository Coordinating Center selected

Biorepository and Registry launched 2021

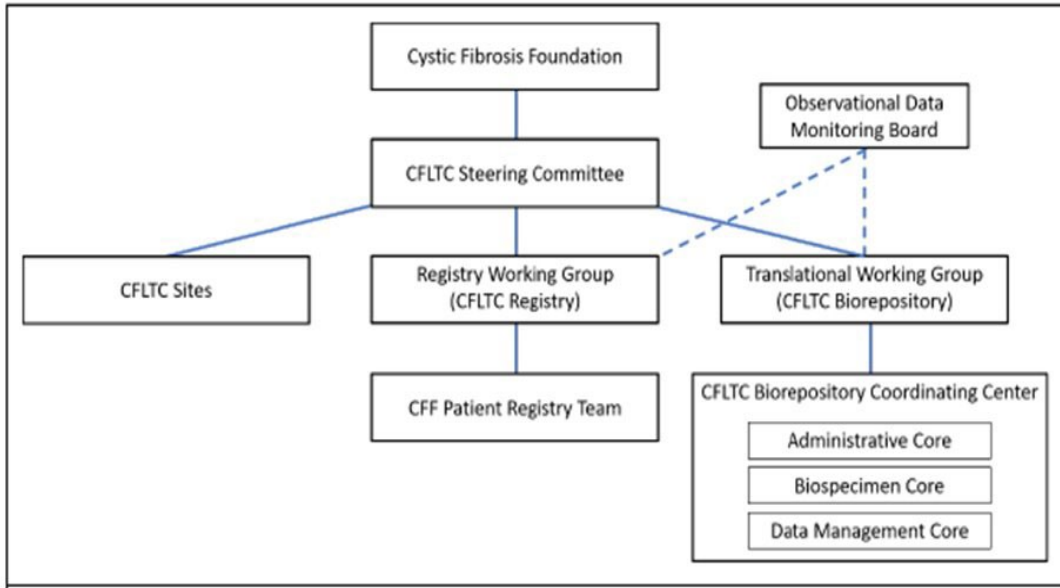
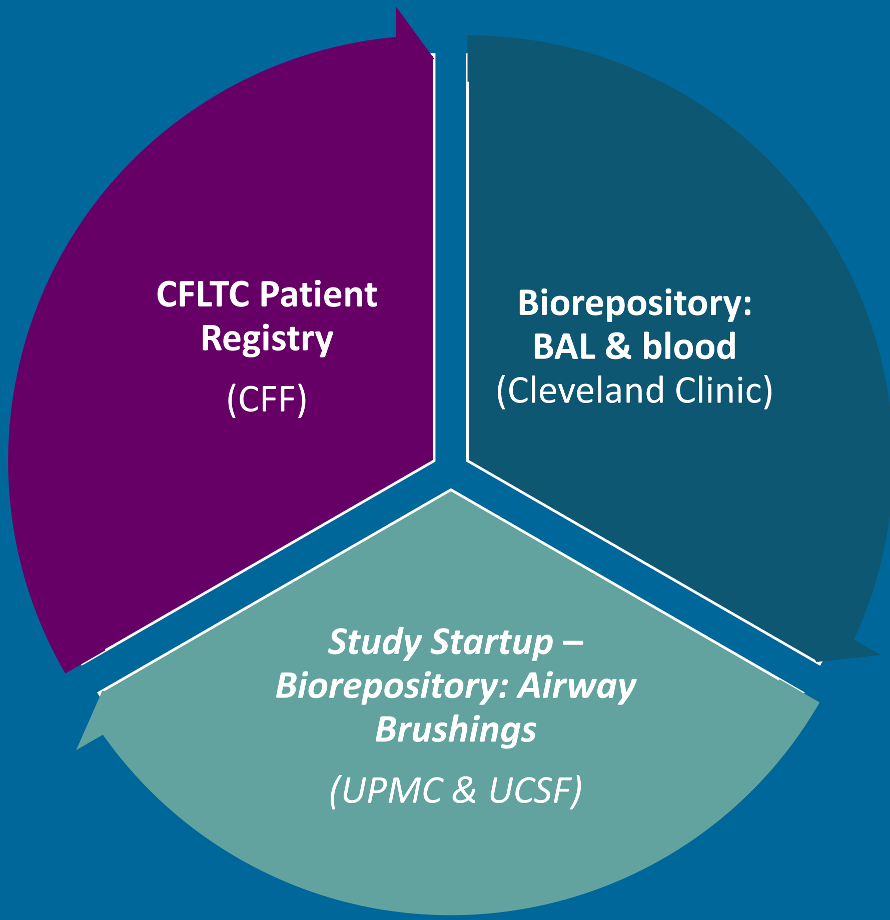


Fig. 1. Structure of Cystic Fibrosis Lung Transplant Consortium (CFLTC)

Research Highlights: Biorepository/Registry

Aim: Identify important predictors of CLAD through collection of biospecimens linked to post-transplant clinical data

1,000 participants enrolled to date



CFLTC Patient Registry

(CFF)

Biorepository: BAL & blood

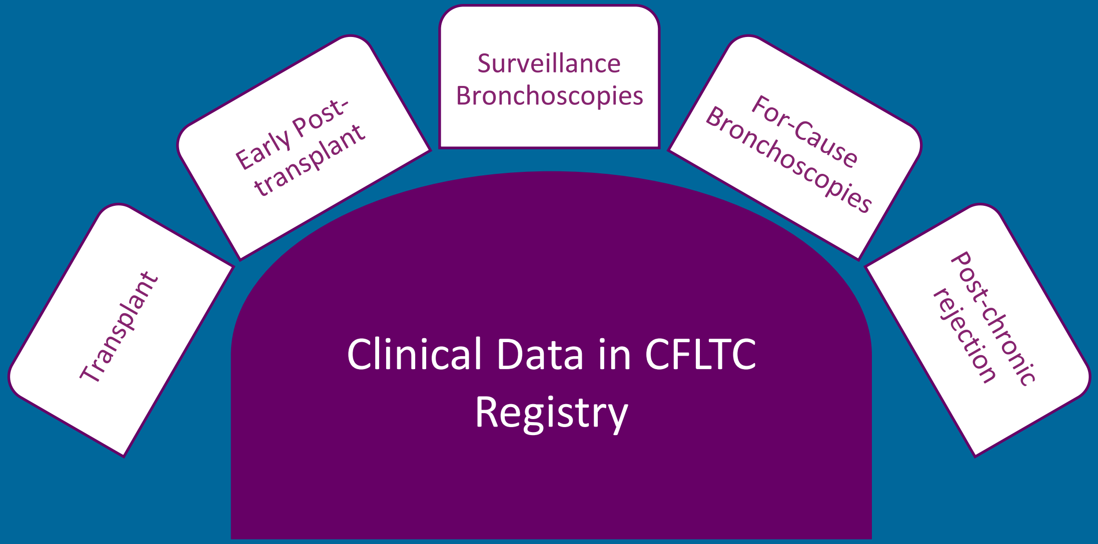
(Cleveland Clinic)

Study Startup – Biorepository: Airway Brushings

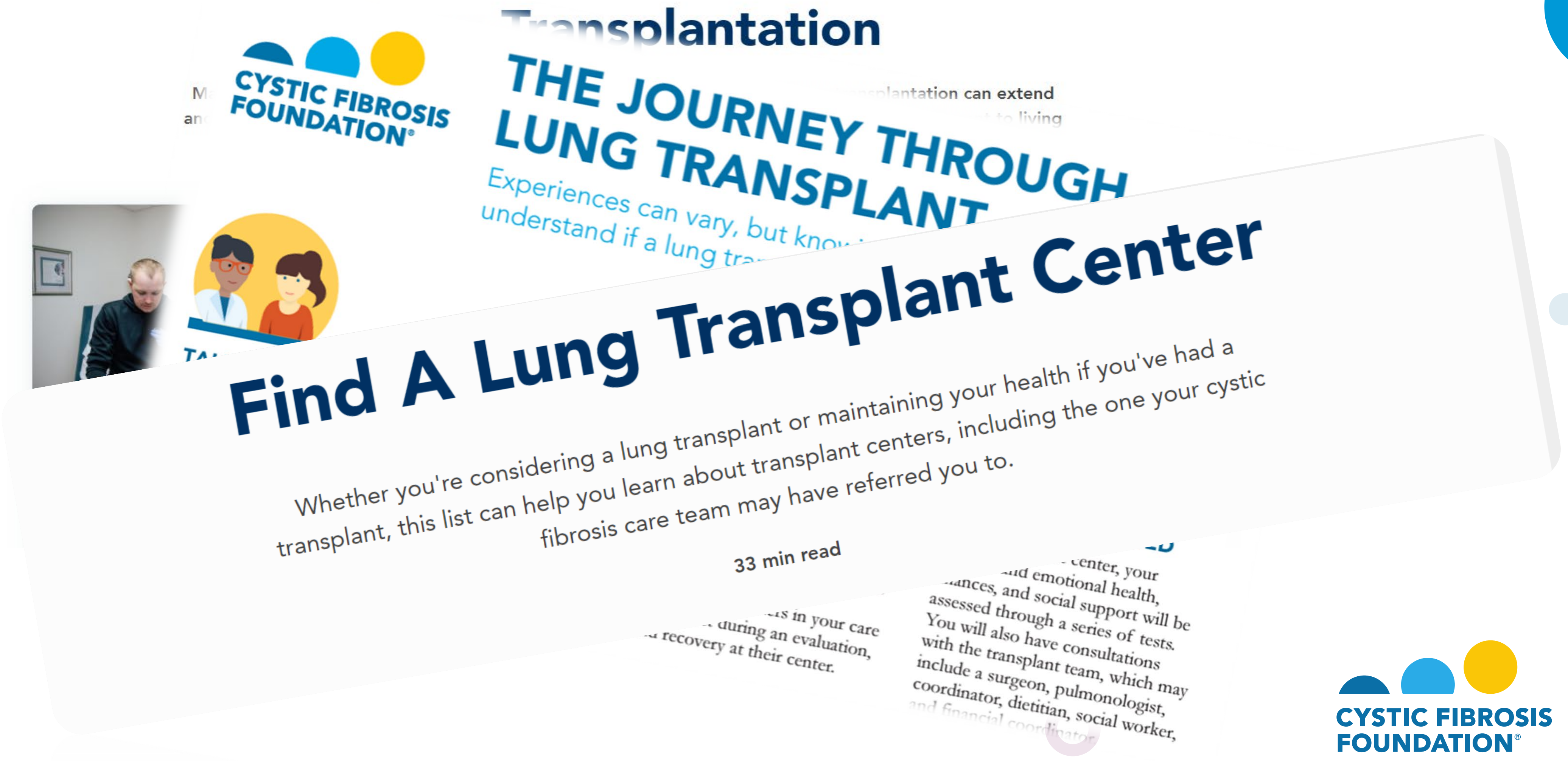
(UPMC & UCSF)

Research Highlights: CFLTC Registry

- **Aim:** To work in tandem with the Biorepository with a focus on **post-transplant** variables related to Chronic Lung Allograft Dysfunction (CLAD)
- Data entered by lung transplant program (Consortium sites)
- 3:1 Match Enrollment: 2 non-CF for every CF patient enrolled
- Separate from CFF Patient Registry, but data will be linked for people with CF



Patient Education and Transplant Resources



Transplantation

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THE JOURNEY THROUGH LUNG TRANSPLANT

Find A Lung Transplant Center

Whether you're considering a lung transplant or maintaining your health if you've had a transplant, this list can help you learn about transplant centers, including the one your cystic fibrosis care team may have referred you to.

33 min read

assessed through a series of tests. You will also have consultations with the transplant team, which may include a surgeon, pulmonologist, coordinator, dietitian, social worker,

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https://www.cff.org/managing-cf/lung-transplantation

Key takeaways:

- Support the development of a high-quality specialized network
  - Take time to build relationships and trust within the network
- Patient registry has been instrumental in defining disease trajectory, comparing outcomes, and facilitating research
- Standardize approaches where it matters
  - Define best practices and disseminate them
- Include Quality Improvement
- Support a clinical research infrastructure within a specialized network
- Encourage and cultivate multicenter collaboration
- Give patient’s and families a seat at the table

UNTIL IT'S DONE.

CURE CF!