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Lessons Learned: The Evolution of CF Care

Disclosures

Albert Faro has no personal disclosures relevant to today's presentation.

However, to advance drug development and a search for a cure, his employer the Cystic Fibrosis Foundation (CFF) has contracts with several companies to help fund the development of potential treatments and/or cures for cystic fibrosis. Pursuant to these contracts, CFF may receive milestone-based payments, equity interests, royalties on the net sales of therapies, and/or other forms of consideration. Resulting revenue received by CFF is used in support of our mission. See "How Drugs Get on the Pipeline" on the CFF website for more information.

Additionally, CFF may license CFF Patient Registry data to some companies to monitor drug safety as part of the U.S. Food and Drug Administration's required Phase 4 clinical trials process and to encourage research aimed at improving the care of people with CF, while maintaining our obligation and commitment to protect the privacy of Registry participants. In connection with these licenses, and upon request, CFF may also assist company researchers in interpreting CFF Patient Registry data to aid in designing, analyzing, and interpreting real world studies in CF.

The National Cystic Fibrosis Research Foundation - 1955

Mission:

To assure the development of the means to cure and control CF

CYSTIC FIBROSIS FOUNDATION
ADDING TOMORROWS

Cornerstones Set in Place: 1960's

- CF Care Center Network

  Benefits:

  1) Interdisciplinary care
  2) Descriptive research
  3) Exposure to faculty and trainees

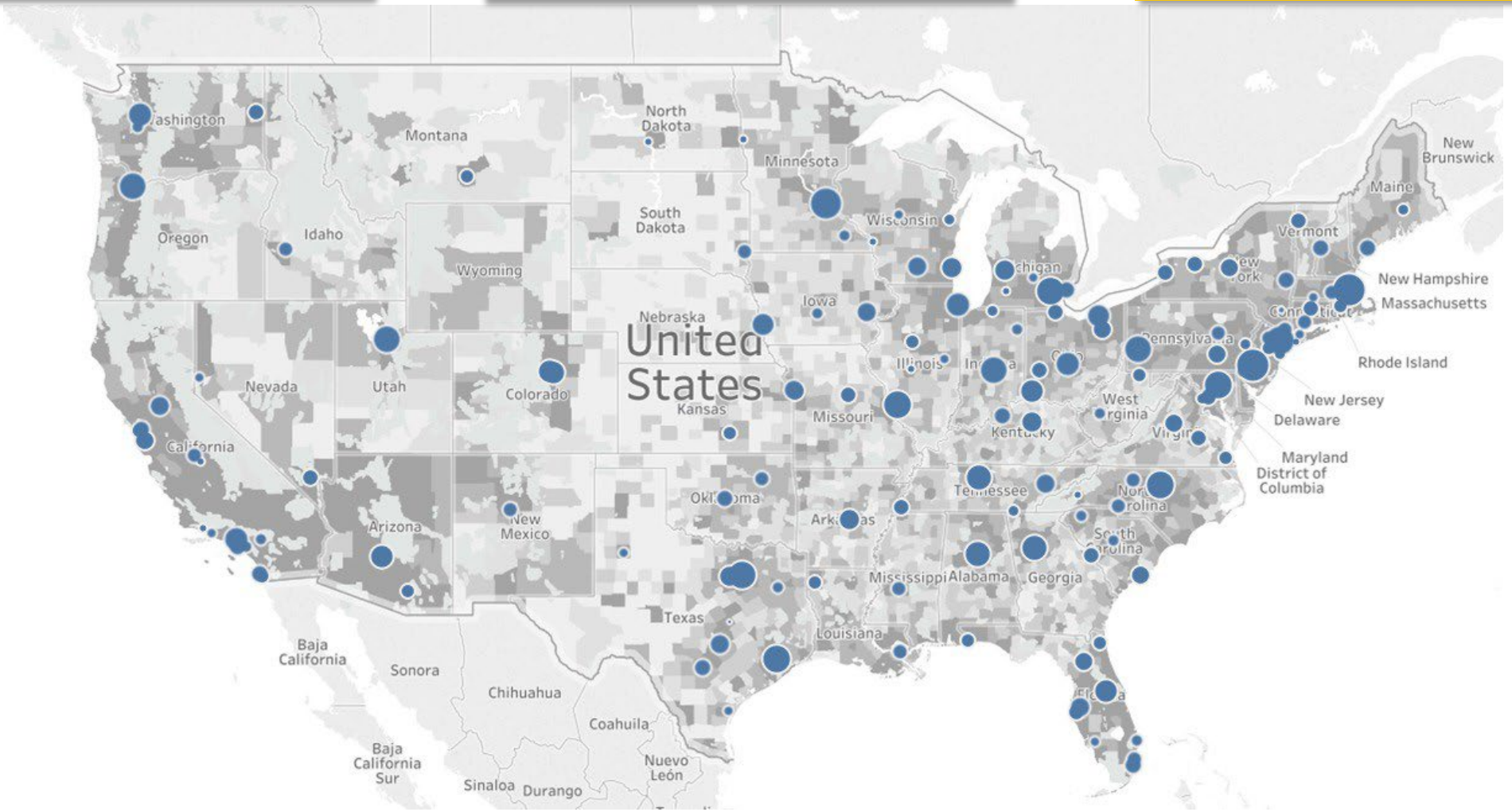
- Patient Registry

  Benefits:

  1) Natural history of the disease
  2) Track the impact of interventions

CF Foundation Care Center Network

Pediatric Programs	Adult Programs	Affiliate Programs
132	119	35



Care Centers Staffed by Interdisciplinary Teams

Practice informed by CF Foundation Clinical Care Guidelines

US Cystic Fibrosis Foundation and European Cystic Fibrosis Society consensus recommendations for the management of non-tuberculous mycobacteria in individuals with cystic fibrosis

Cystic Fibrosis Foundation Pulmonary Guidelines
Use of Cystic Fibrosis Transmembrane Conductance Regulator Modulator Therapy in Patients with Cystic Fibrosis

International Committee on Mental Health in Cystic Fibrosis: Cystic Fibrosis Foundation and European Cystic Fibrosis Society consensus statements for screening and treating depression and anxiety

Models of Palliative Care Delivery for Individuals with Cystic Fibrosis: Cystic Fibrosis Foundation Evidence-Informed Consensus Guidelines

Cystic Fibrosis Foundation consensus guidelines for the care of individuals with advanced cystic fibrosis lung disease

High-Quality Specialized Care: Patient Registry

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

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

CYSTIC FIBROSIS FOUNDATION
ADDING TOMORROWS

Uses of the Cystic Fibrosis Foundation Patient Registry

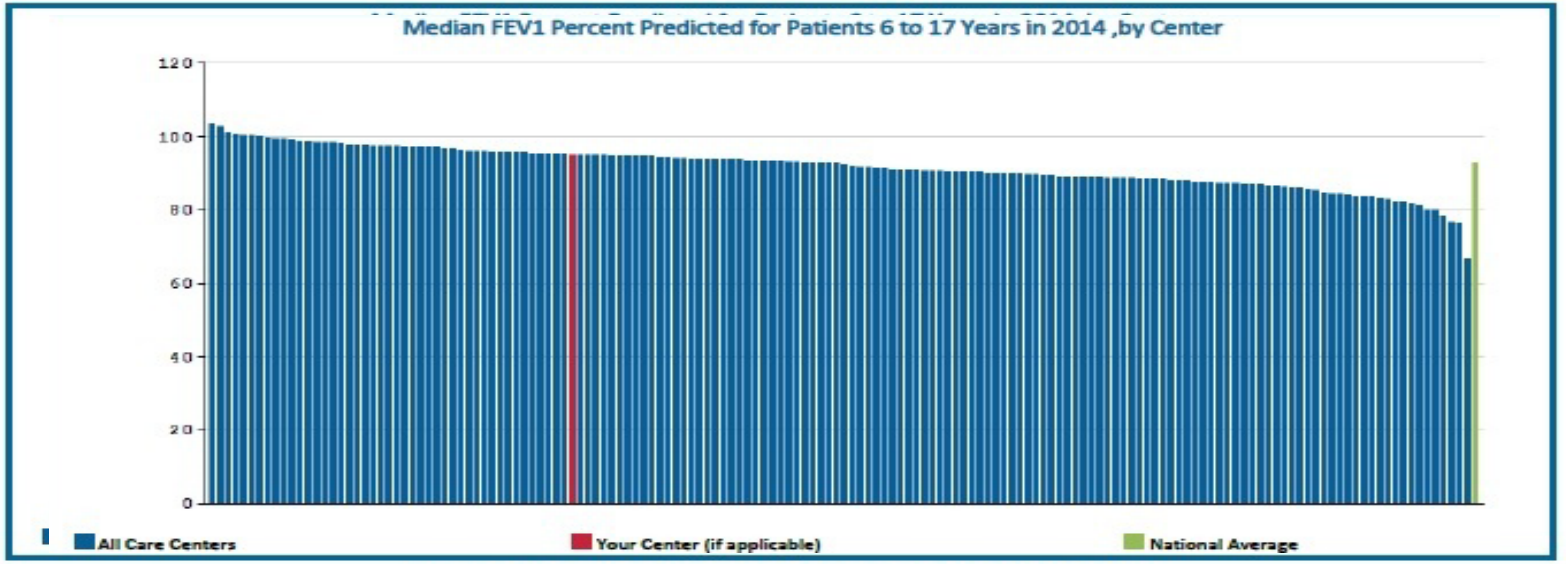
DISEASE SURVEILLANCE	FRAMEWORK FOR CLINICAL TRIALS	POST-MARKETING SURVEILLANCE STUDIES	QUALITY IMPROVEMENT	COMPARATIVE EFFECTIVENESS RESEARCH
				
Track progress in curing CF and the impact of treatments	Test promising new therapies	Ensure safety and effectiveness of approved products	Provide all patients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

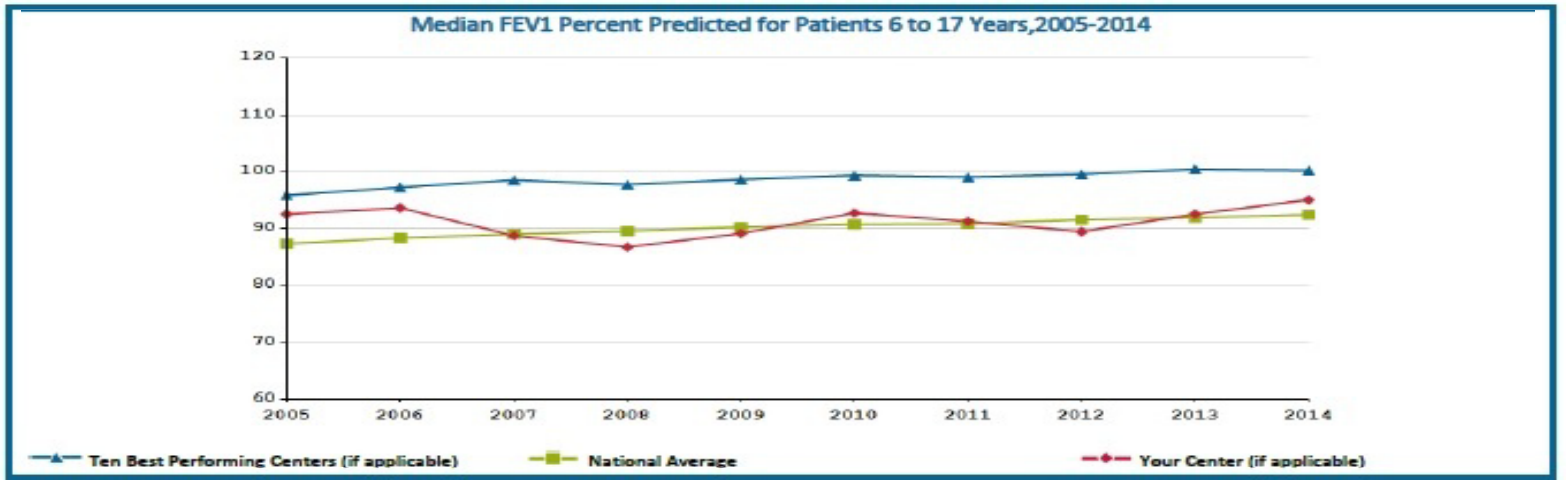
CYSTIC FIBROSIS FOUNDATION
ADDING TOMORROWS

Annual Center-specific reports

Median FEV1 Percent Predicted for Patients 6 to 17 Years

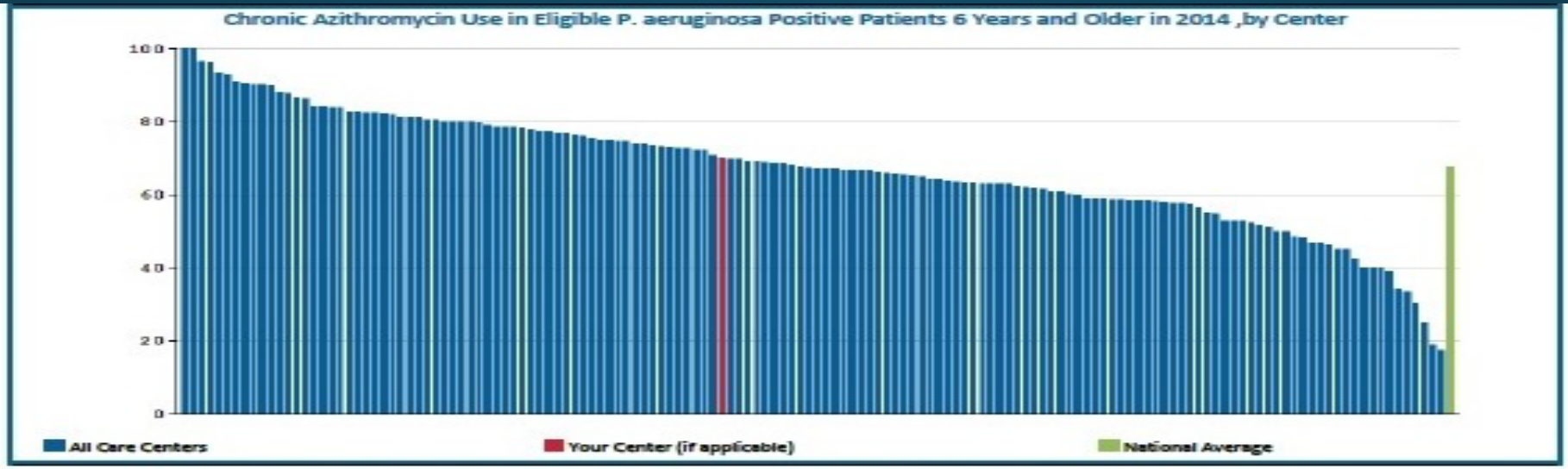
Center Name , 2014	95.1
Care Center Network - Median of All Center Values, 2014	92.5
Care Center Network - Average of Top 10 Center Values, 2014	100.3
National Average of All Patients, 2014	92.5

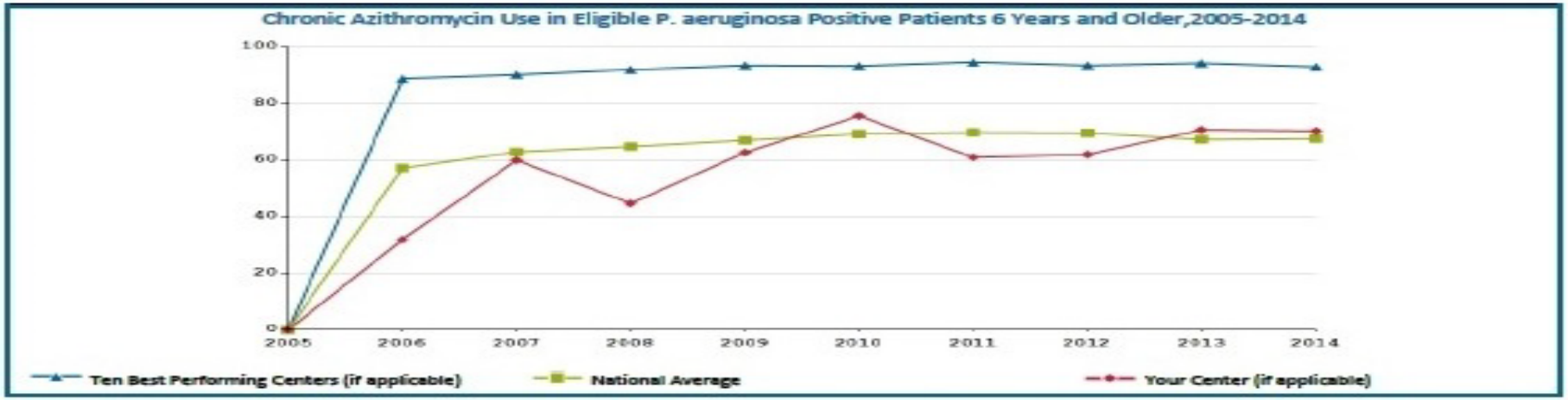




CFFP Center-Specific Report

CYSTIC FIBROSIS FOUNDATION
ADDING TOMORROWS

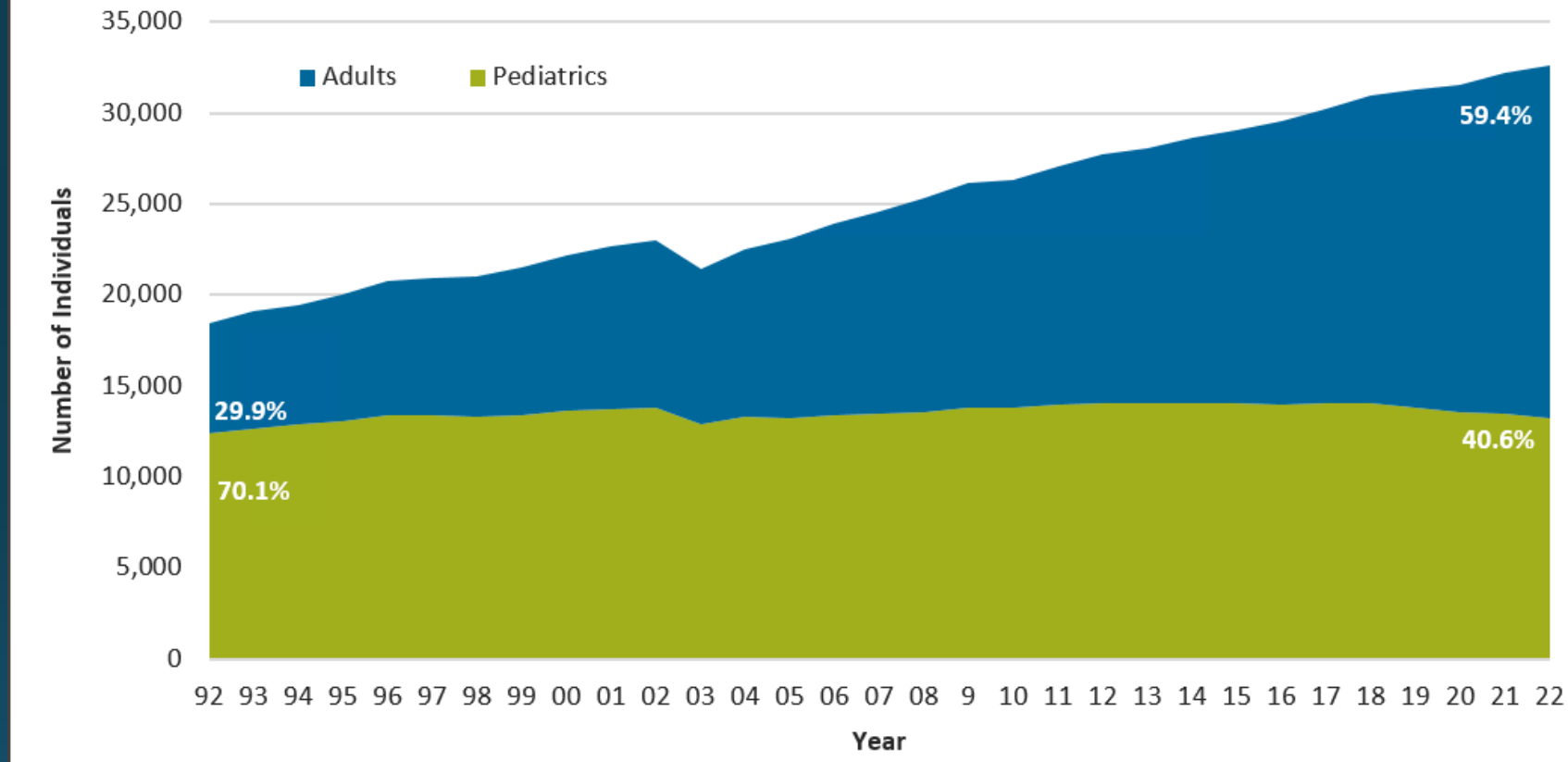




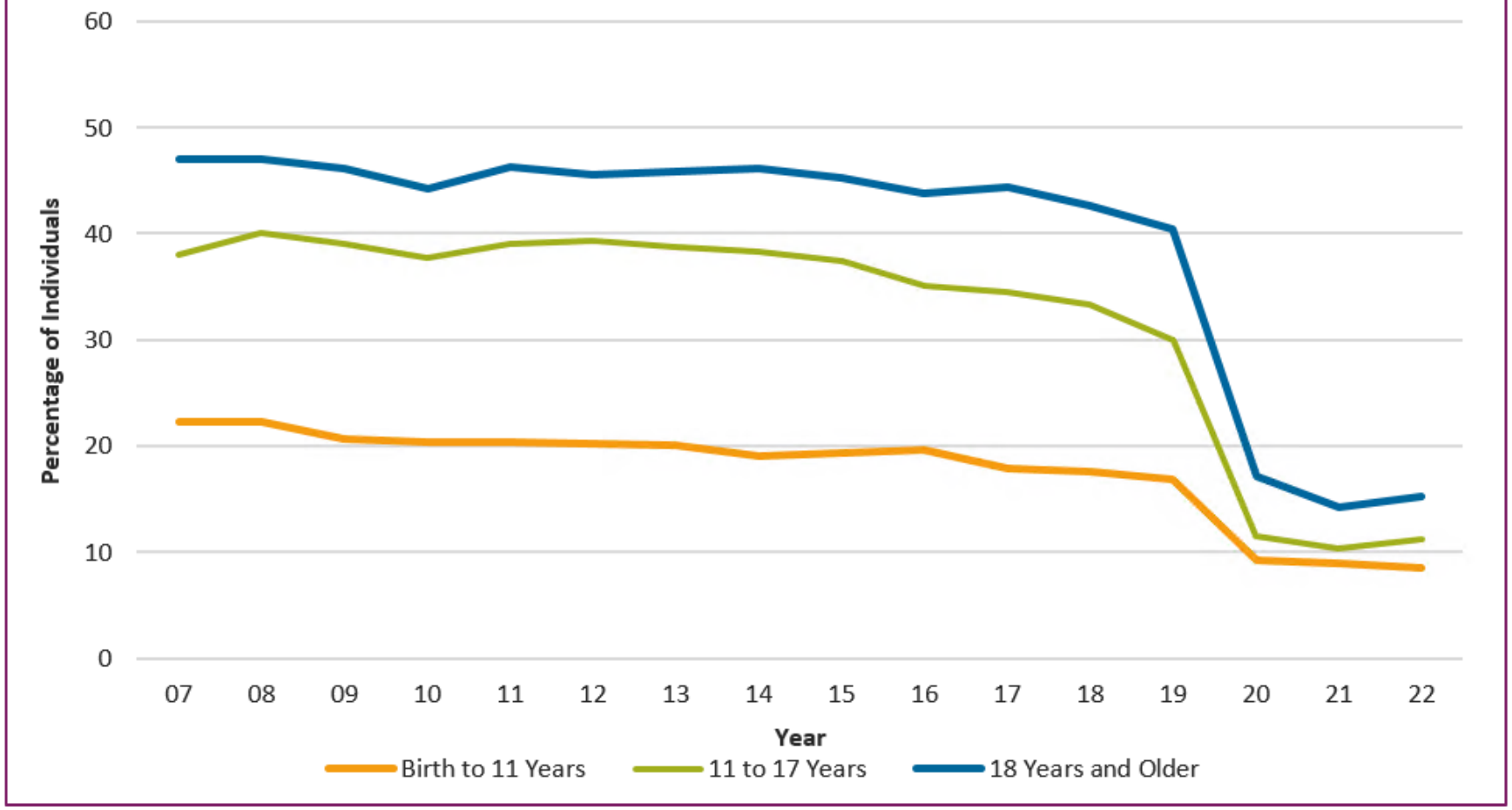
	2005	2006	2007	2008	2009	2010	2011	2012	2013	2014
Your Center	0.0	31.9	60.0	44.7	62.7	75.6	61.0	62.0	70.7	70.3
National Average	0.0	57.2	62.9	64.8	67.0	69.3	69.8	69.6	67.3	67.6
Ten Best Performing Centers	0.0	88.8	90.2	92.0	93.5	93.3	94.7	93.5	94.3	93.0

CYSTIC FIBROSIS FOUNDATION
ADDING TOMORROWS

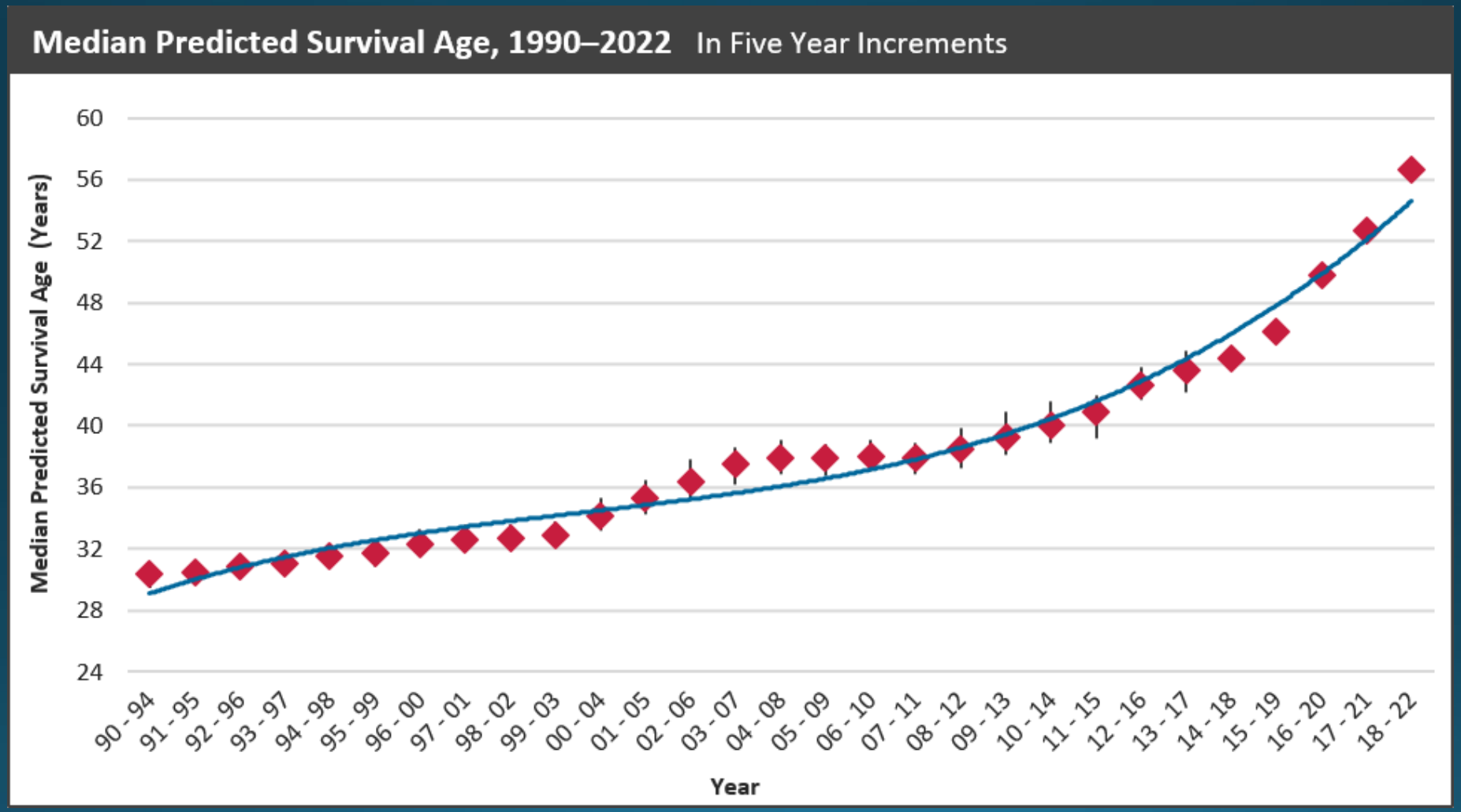
Number of Children and Adults with CF, 1992–2022



Individuals Treated with IV Antibiotics for a Pulmonary Exacerbation, 2007–2022



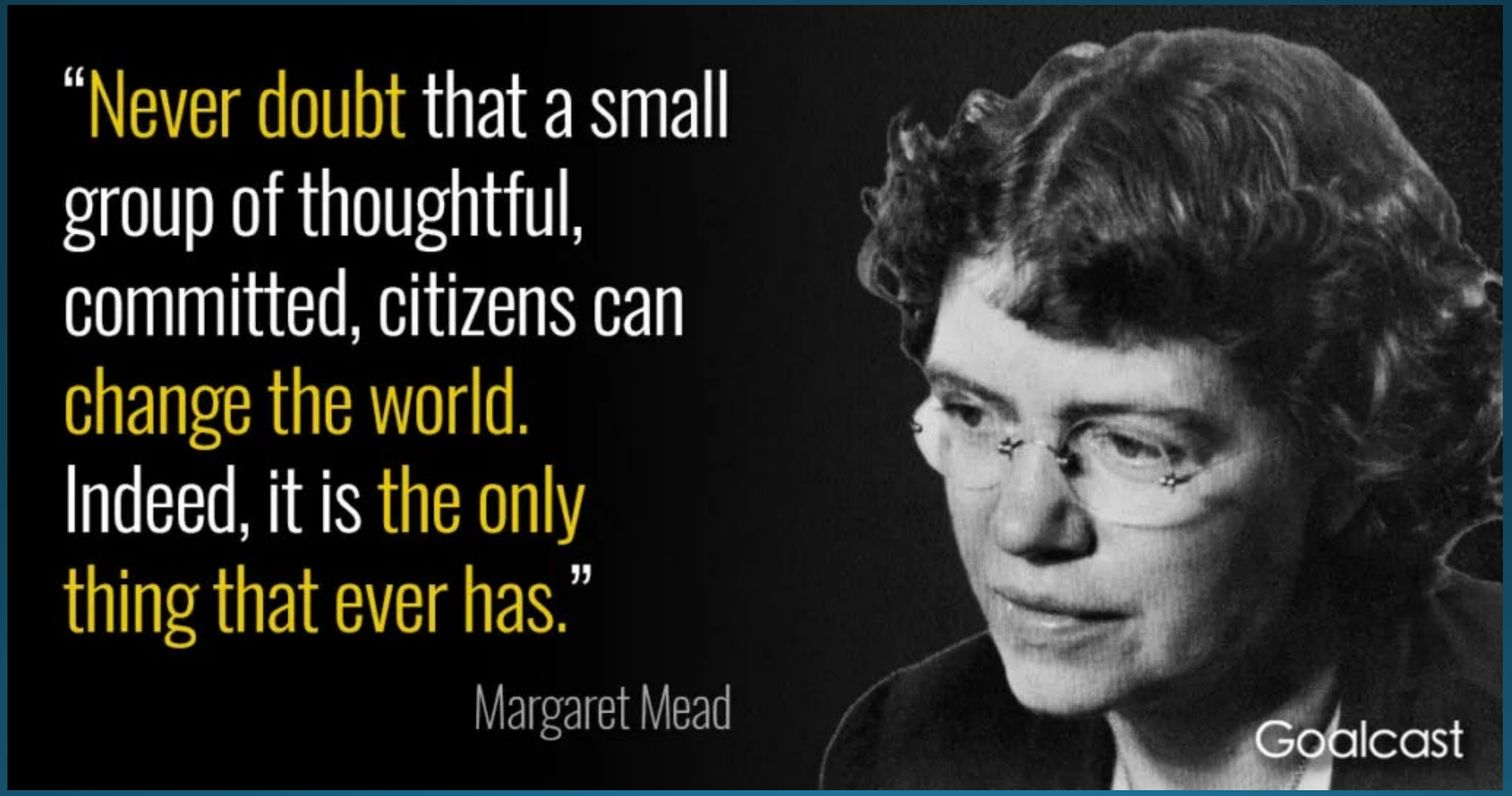
Survival


Median Predicted Survival Age, 1990–2022 In Five Year Increments
Median Predicted Survival Age (Years)
60
56
52
48
44
40
36
32
28
24
90 - 94
91 - 95
92 - 96
93 - 97
94 - 98
95 - 99
96 - 00
97 - 01
98 - 02
99 - 03
00 - 04
01 - 05
02 - 06
03 - 07
04 - 08
05 - 09
06 - 10
07 - 11
08 - 12
09 - 13
10 - 14
11 - 15
12 - 16
13 - 17
14 - 18
15 - 19
16 - 20
17 - 21
18 - 22
Year

In order to accelerate improvement in outcomes for people with ALS:

- Support the development of high-quality specialized care centers
- Establish a patient registry to define disease disease trajectory, compare outcomes, and facilitate research
- Define best practices and disseminate them
- Standardize approaches where it matters
- Train sites in QI
- Promote benchmarking visits
- Support a clinical research infrastructure within the care center network

Summary


"Never doubt that a small group of thoughtful, committed, citizens can change the world. Indeed, it is the only thing that ever has."
Margaret Mead
Goalcast

UNTIL IT'S DONE.

CURE CF!