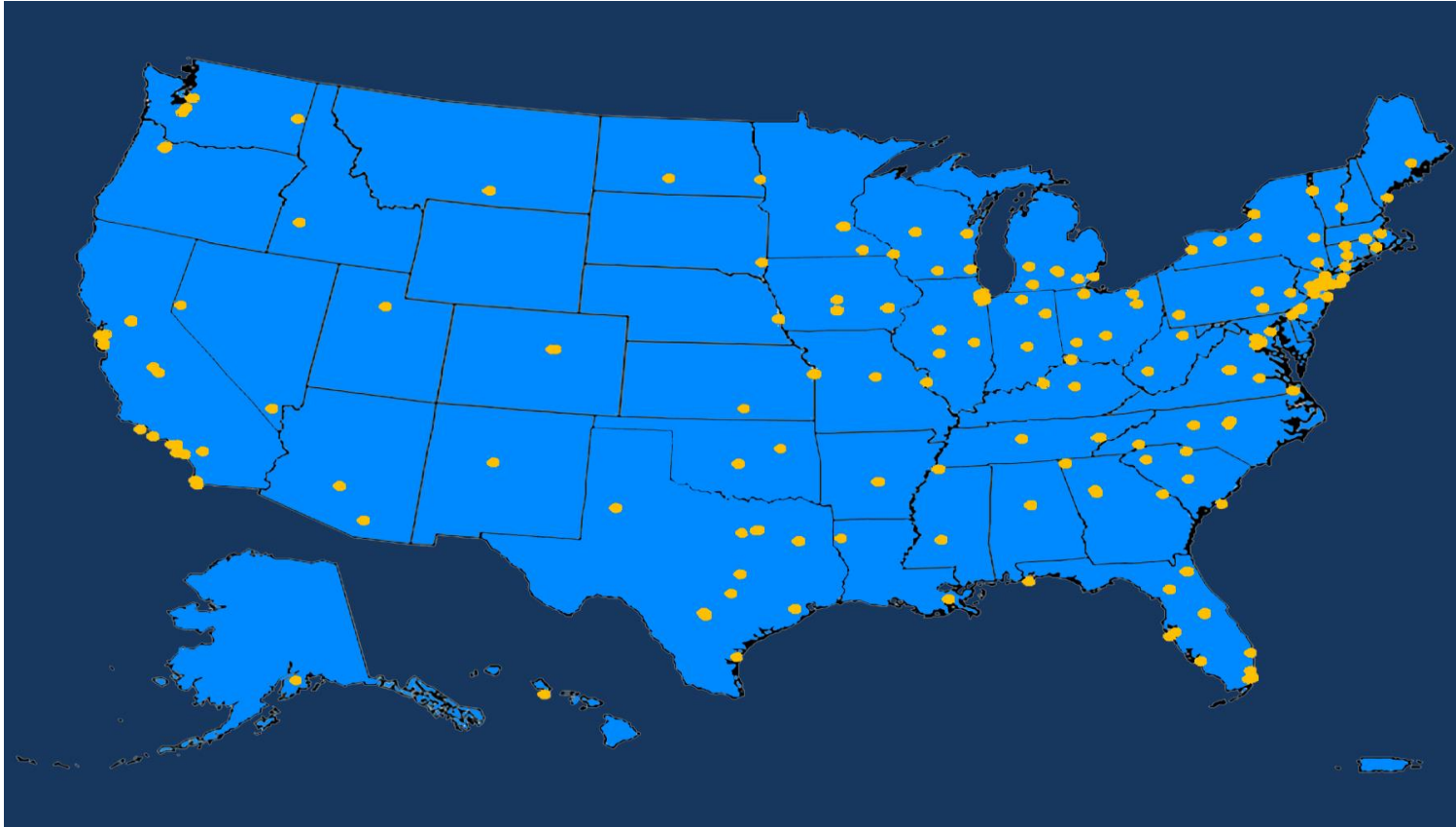




Overview of CFF and Challenges ~~They~~ We Face

Alexander Elbert
Senior Director, Patient Registry, CFF, USA



The CF Foundation sustains a network of 121 accredited CF care centers (comprised of 121 pediatric care programs, 105 adult care programs and 51 affiliate programs) across the United States.

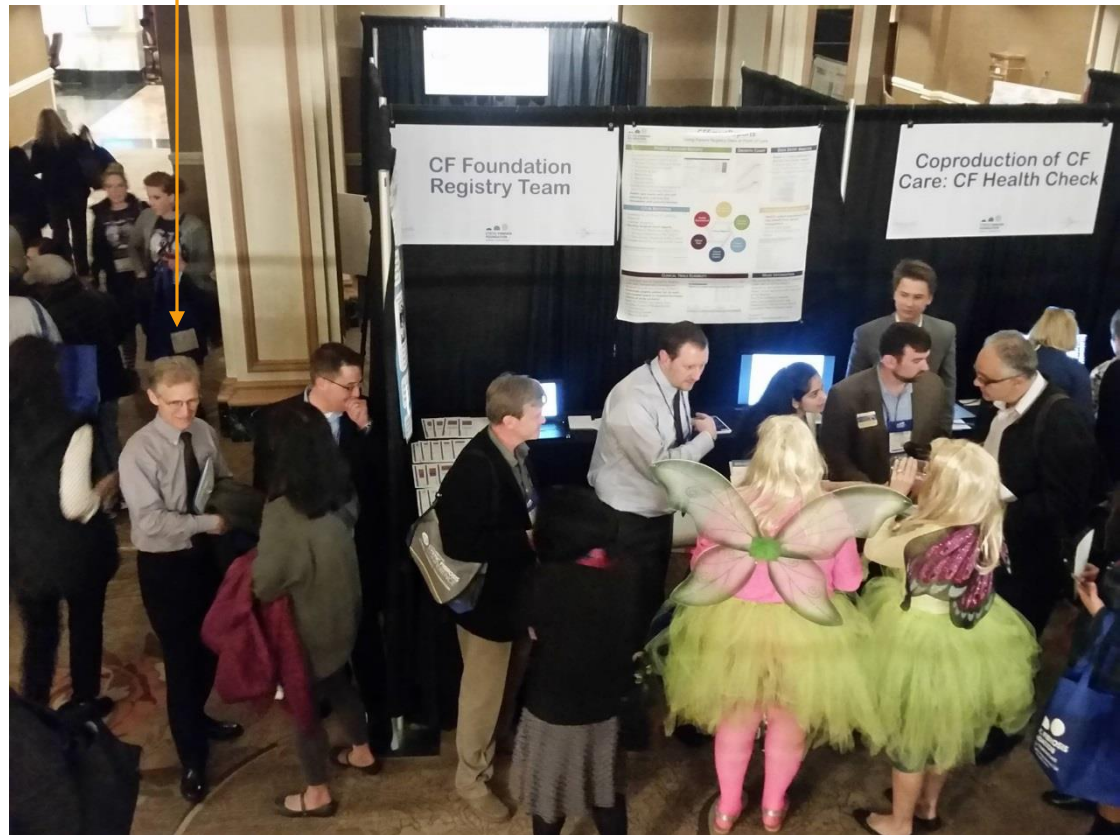
Minimum Requirement

- CF Center Director
- CF Center Associate Director
- CF Center Coordinator
- SW, Nurse, Dietician, RT, PT, administrator
- Recommended: psychologist, pharmacist
- Involvement of: Endocrinologist, GI, ID (infectious disease)

Important: Accredited CF center must participate in CF Patient Registry

Meet the Registry Team

Clinical Affairs department is headed by
Dr. Bruce Marshall, SVP of CFF

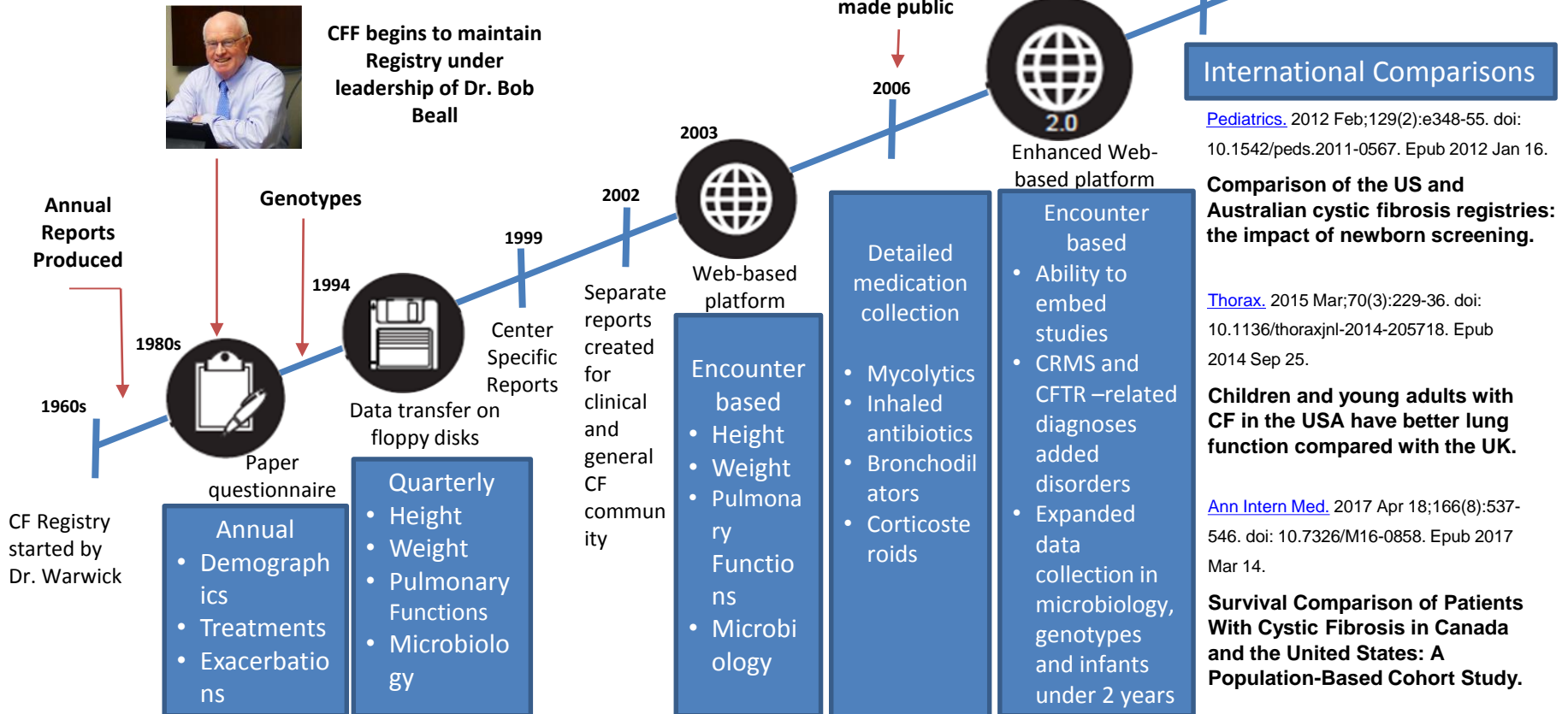


Freshly published 2015 report!

QI Fair at NACFC 2017

US CF Registry Timeline of Events

YEAR	1985	1990	1995	2000	2005	2010	2015	2016
# Patients	15,200	17,600	20,100	22,200	23,200	26,400	29,100	29,600
FEV1 %, median	60.7	65.7	70.8	73.5	76.8	79.0	80.3	80.9
% Adults	29	32	35	39	43	47	52	53



“The best registry in America, in my opinion, is the cystic fibrosis registry”



Martin Adel Makary, M.D., M.P.H.

Surgical Director, Johns Hopkins Multidisciplinary Pancreas
Clinic

Professor of Surgery, Interview to National Public Radio, 2015

Uses of CF Registry

DISEASE SURVEILLANCE



Track progress
in curing CF
and the
impact of
treatments

FRAMEWORK FOR CLINICAL TRIALS



Test
promising
new
therapies

POST- MARKETING SURVEILLANCE STUDIES



Ensure safety
and
effectiveness
of approved
products

QUALITY IMPROVEMENT



Provide
all people
with CF
with high
quality care

COMPARATIVE EFFECTIVENESS RESEARCH



Promote
evidence-based
clinical
decision
making

INTERNATIONAL COMPARISONS



Compare
impact of
different
health
systems

- Pre-visit planning
- Predictive analytics
- Recruiting patients for clinical trials

CF Patient Registry Today

Inclusion criteria

- Seen at CF Care Center
- Consent to participate

Data collected at

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

Download a copy at
CFF.org/InsightCF

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



[CF Diagnosis](#)

[Demographics](#)

[Annual Review](#)

[Care Episode](#)

[Encounter](#)

[Note](#)

[File Upload](#)

CF Diagnosis

Diagnosis Information

History of patient diagnosis

Date is an approximation [approxdiagnosisdate]

Date of diagnosis (may be earlier than first sweat test): [diagnosisdate] [precision=3]
DD MMM YYYY

Diagnosis [patient_dx]

Patient was diagnosed with CF after false negative result by newborn screening Yes [1] No [2] Unknown [3] ↻ [newbornscreen]

Note: CRMS cases are identified by positive newborn screening

Diagnosis suggested by the following:

- Acute or persistent respiratory abnormalities [1]
- CBAVD (absent vas deferens) or related abnormalities [2]
- Digital clubbing [3]
- DNA Analysis [4]
- Edema [5]
- Electrolyte imbalance [6]
- Elevated immunoreactive trypsinogen (IRT) at CF newborn screening [7]
- Failure to thrive/malnutrition [8]
- Family history [9]
- Infertility/GU abnormalities [10]
- Less than 2 identified disease causing mutations [11]
- Liver problems [12]
- Meconium ileus/other intestinal obstruction (provide details below) [13]

The Cystic Fibrosis Foundation Patient Registry

Design and Methods of a National Observational Disease Registry

Emily A. Knapp¹, Aliza K. Fink¹, Christopher H. Goss², Ase Sewall³, Josh Ostrenga¹, Christopher Dowd¹, Alexander Elbert¹, Kristofer M. Petren¹, and Bruce C. Marshall¹

¹Cystic Fibrosis Foundation, Bethesda, Maryland; ²Department of Medicine, University of Washington, Seattle, Washington; and ³Sewall, Inc., Bethesda, Maryland

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**

CF Registry at a glance in 2017

Data on 30,000 patients was entered by more than 700 people from 124 sites;

It included:

- 197,000 + Encounters (including annual);**
- 24,000 + Home or Hospital IV treatments;**
- 890 + New CF Diagnoses;**
- 570+ Newborns with CF;**
- 1,500 + Lung Transplant patients.**

**Each CF patient had, on average, 4.4 encounter records in the registry.
Each patient's record could have up to ~850 raw data elements available to describe health and socio-economic status**

Registry has data on more than 50,000 patients since 1982!

CF Patient Registry Web Platform (PortCF) strengths



Strengths

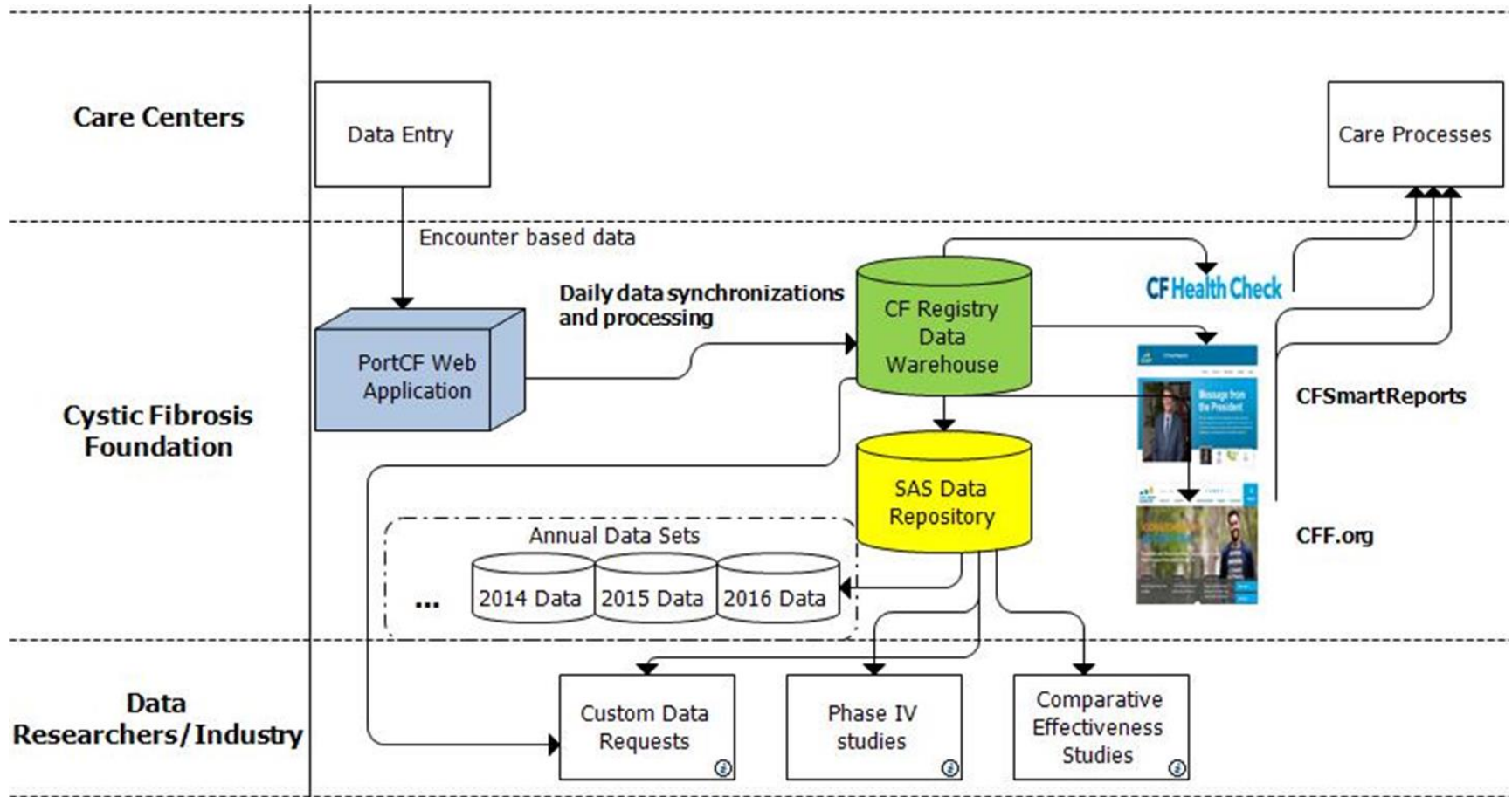
1. Stable and well learned application
2. Mature processes
3. Well thought out registry platform
4. Built-in features to improve data quality



Weaknesses

1. Code and application is not owned by CFF
2. Maintenance and changes are expensive
3. Reporting tools need improvement, ...
and we don't want to improve them .
4. Obsolete underlying technology
5. Manual data entry

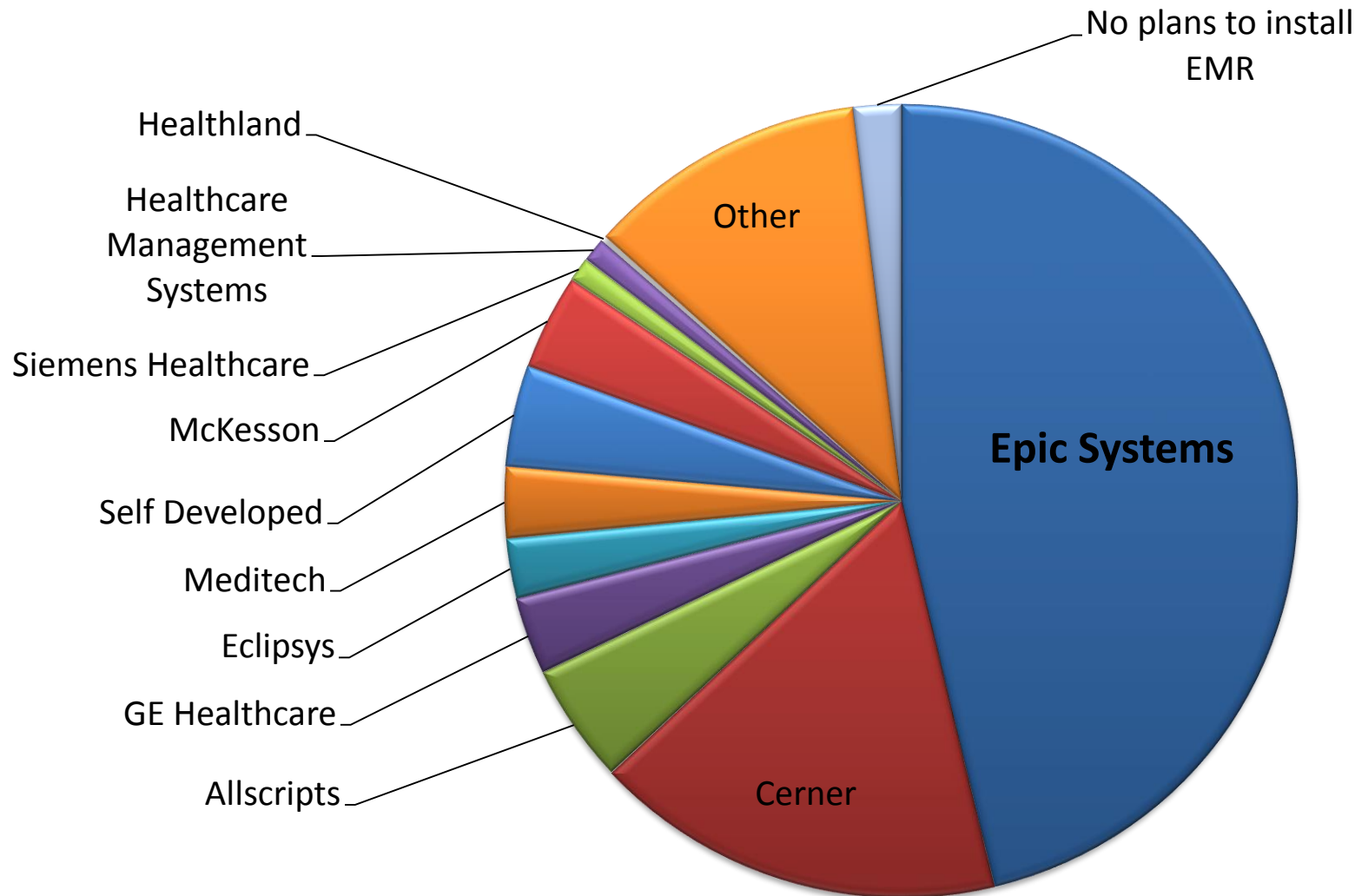
Registry Data Flow



Why we still don't use EMR – registry data exchanges

1. Not all data elements that we collect exist in EMR. Examples: classes of antibiotics; participation in observational and interventional studies, assistance from a Patient Assistance Program, mother/father education, etc.
2. In-patient and out-patient data could be stored in different EMRs
3. Different levels of local IT support to CF care teams
4. Necessity to maintain PortCF and EMR- Registry interfaces simultaneously;
5. Established data flows for Phase IV studies

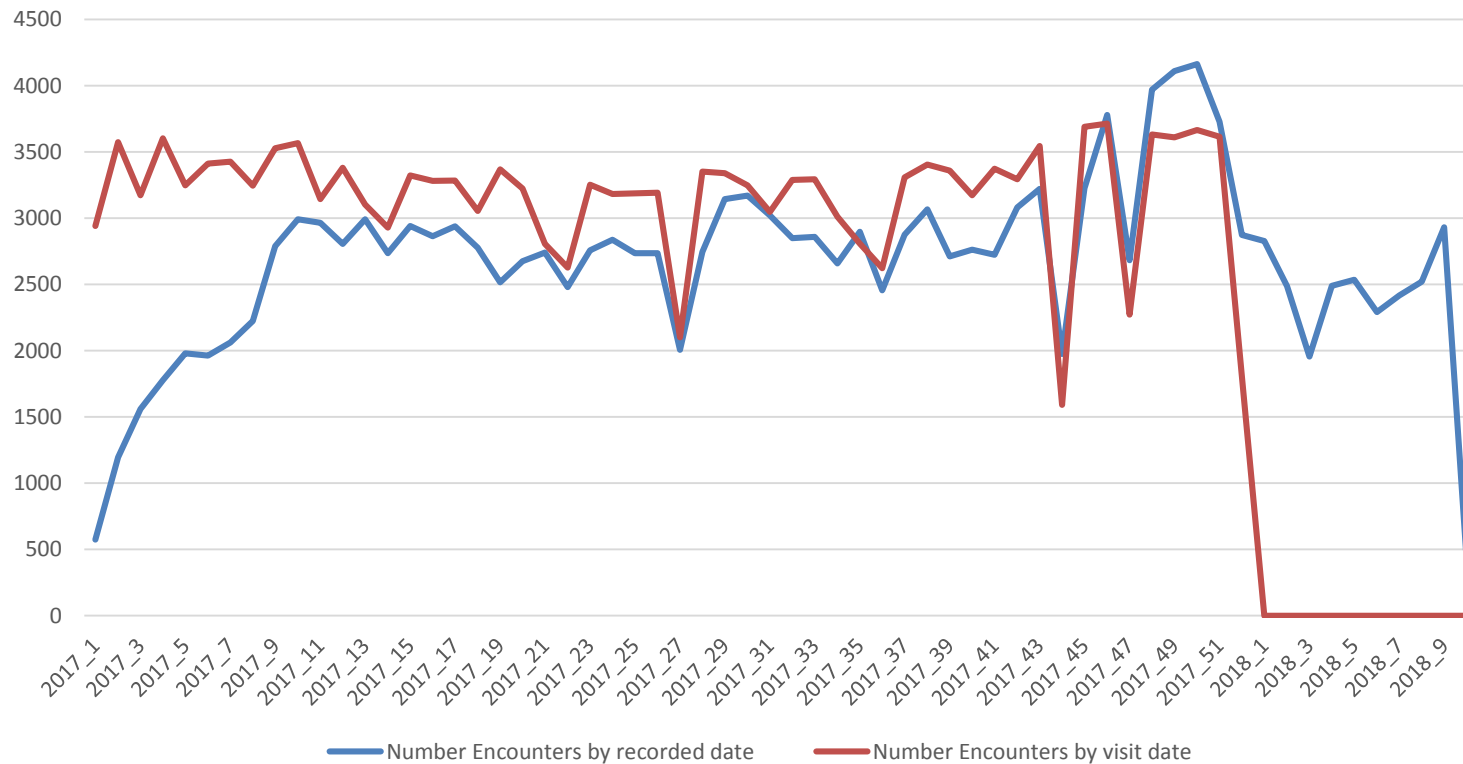
EMR Landscape in CF Care Centers, 2013



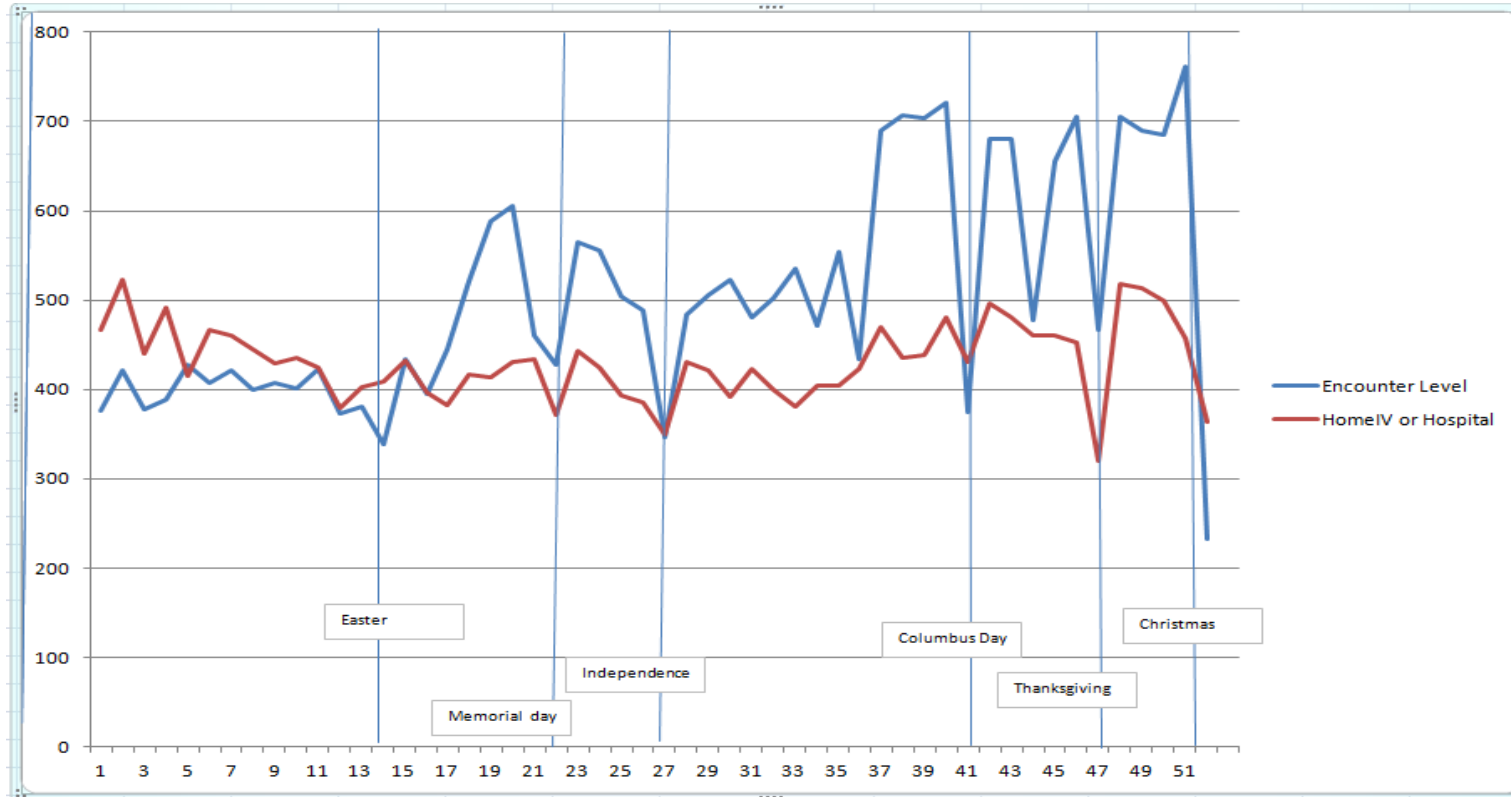
Timeliness of Data Collection

Data entry is not evenly distributed across the year

Number of clinic encounters, by encounter date and date of entry to PortCF



Seasonality of clinic visits and exacerbations

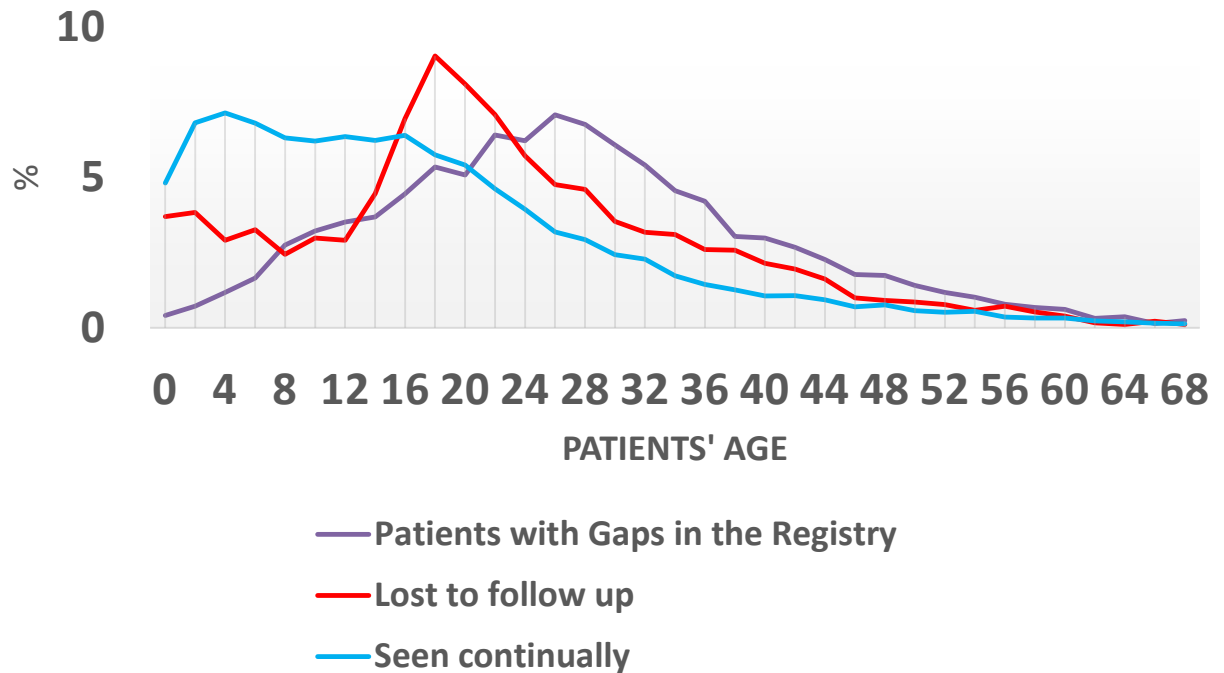


Week of a year

Other Known Issues

- Patients lost to follow up;
- Medications: prescribed vs taken;
- Height and weight errors;
- Adherence and social issues

Age distribution in different cohorts of US patients




Impact of data collection forms on PortCF data

Making exacerbation question mandatory, increased number of PEX by 25%

Exacerbation Assessment

What was your assessment regarding pulmonary exacerbation at this visit?

Absent [1] Mild exacerbation [2] Moderate exacerbation [3] Severe exacerbation [4] Don't know/unable to answer [5]

 [pe_assessment]

**Removing carry forward functionality decreased number of reported hemoptysis cases by almost 100%
In 2015**

Hemoptysis [4]

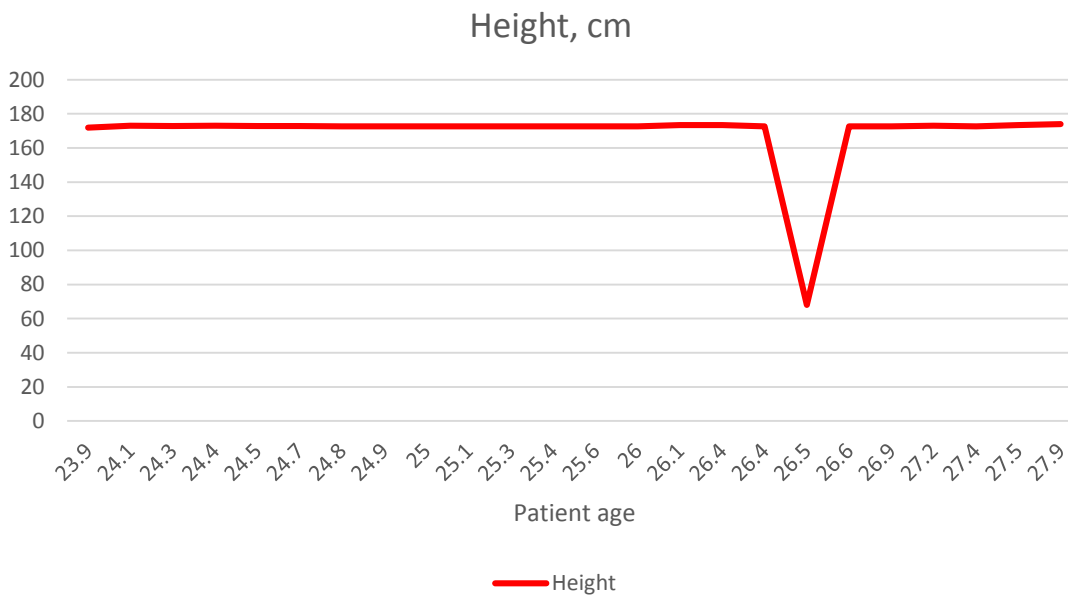
Please specify selection of hemoptysis:

Hemoptysis, massive [1]

Hemoptysis, other [2]

Methods to improve/monitor quality of registry data

- Data verifications with CF Centers, e.g. death dates, transplant dates, hemoptysis;
- Analytical methods to identify wrong values, e.g. wrong height, weight, date of birth, gender, etc;
- User reported errors identified due to QI projects;
- De-duplication of records;
- Programs like MAP (genotyping)
- Recoding of user text entries



Example of height error

- Compares data between EMRs and PortCF;
- Sites are selected randomly and “For-cause”;
- Auditors compare medications, hospitalizations, microbiology
- Excellent matching between EMRs and CF registry for all variables;
- “For-cause” and non TDN sties make slightly more errors
-

Encounter has been reviewed: Encounter not able to be verified: [Encounter List](#)

Demographics/General | Hospitalizations | Vitals | Microbiology | Pulmonary Meds | Enzymes | PFTs | Missing Encounters

Was the following present in missing encounters?	Was found?	Auditor Comments
TOBI	<input type="checkbox"/>	
Bethkis	<input type="checkbox"/>	
Aztreonam (Cayston)	<input type="checkbox"/>	
azithromycin	<input type="checkbox"/>	
Clarithromycin	<input type="checkbox"/>	
Dnase (Dornase alfa)	<input type="checkbox"/>	
Hypertonic saline	<input type="checkbox"/>	
Kalydeco (Ivacaftor)	<input type="checkbox"/>	
MSSA	<input type="checkbox"/>	
MRSA	<input type="checkbox"/>	
Haemophilus influenzae	<input type="checkbox"/>	

	Data Completeness			Data Accuracy - Exclude Height/Weight variables			Data Accuracy - Sites Collecting H/W		
		Mean	P-value	N	Mean	P-value	N	Mean	P-value
	67	0.45	N/A	67	1.51	N/A	54	1.68	N/A
TDN Site			0.025			0.011			0.034
Yes	44	0.26		44	1.32		33	1.49	
No	23	0.56		23	1.86		21	1.96	
For-Cause			0.923			0.031			0.001
Yes	20	0.39		20	1.84		20	2.11	
No	47	0.35		47	1.36		34	1.42	

International comparisons of health and other measures of people with CF may highlight differences in health care and health outcomes between different countries

[Thorax](#). Author manuscript; available in PMC 2016 Apr 20.

Published in final edited form as:

[Thorax](#). 2015 Mar; 70(3): 229–236.

Published online 2014 Sep 25. doi: [10.1136/thoraxjnl-2014-205718](#)

PMCID: PMC4838510

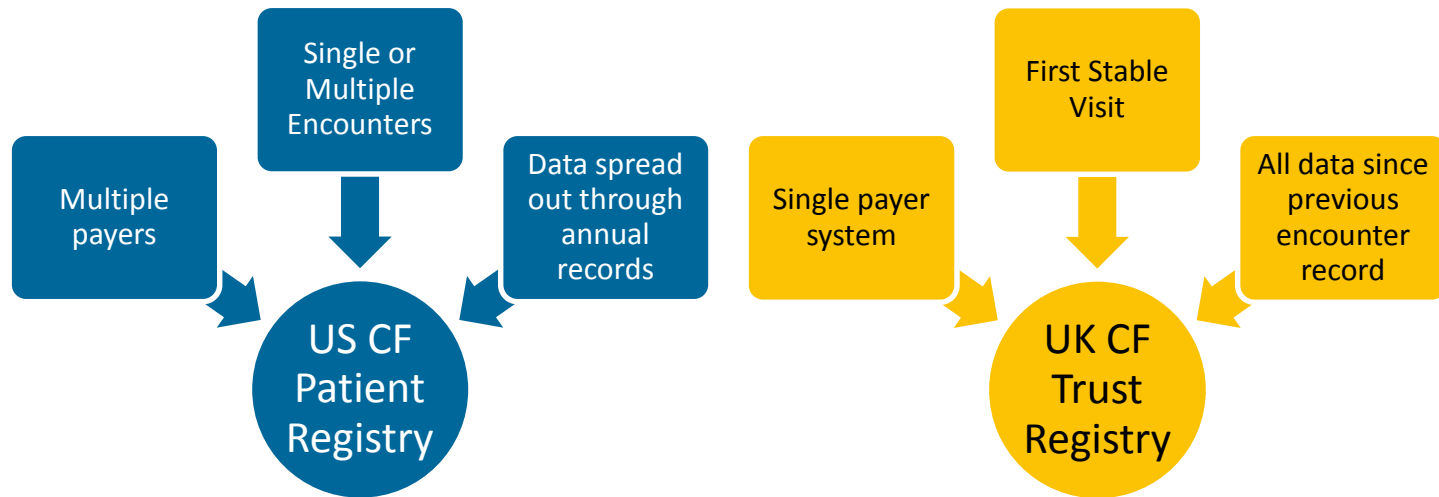
NIHMSID: NIHMS771905

Children and young adults with CF in the US have better lung function as compared to the UK

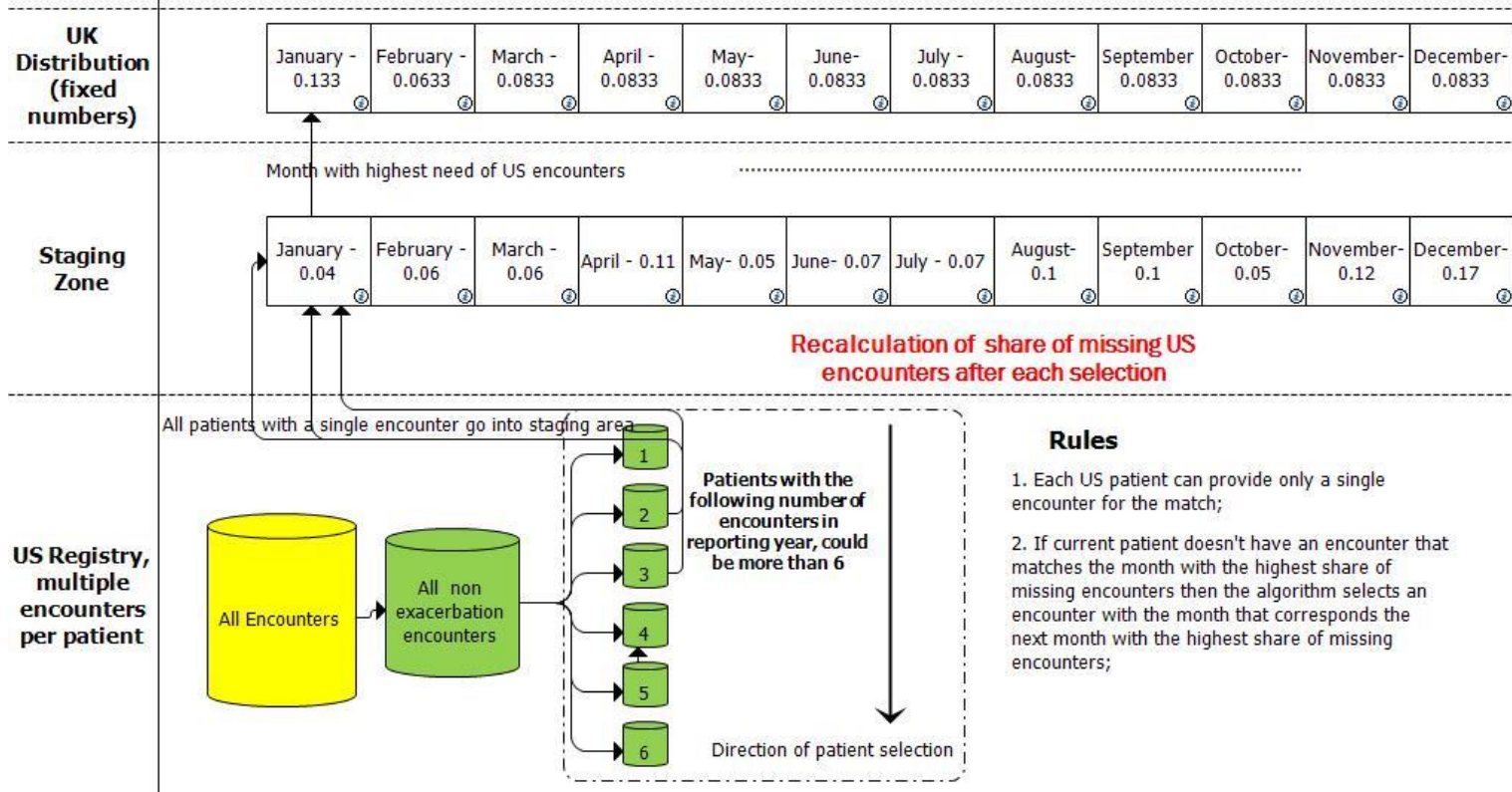
[Christopher H Goss](#),¹ [Stephanie J MacNeill](#),² [Hebe B Quinton](#),³ [Bruce C Marshall](#),⁴ [Alexander Elbert](#),⁴ [Emily A Knapp](#),⁴ [Kristofer Petren](#),⁴ [Elaine Gunn](#),⁵ [Joanne Osmond](#),⁵ and [Diana Bilton](#)⁶

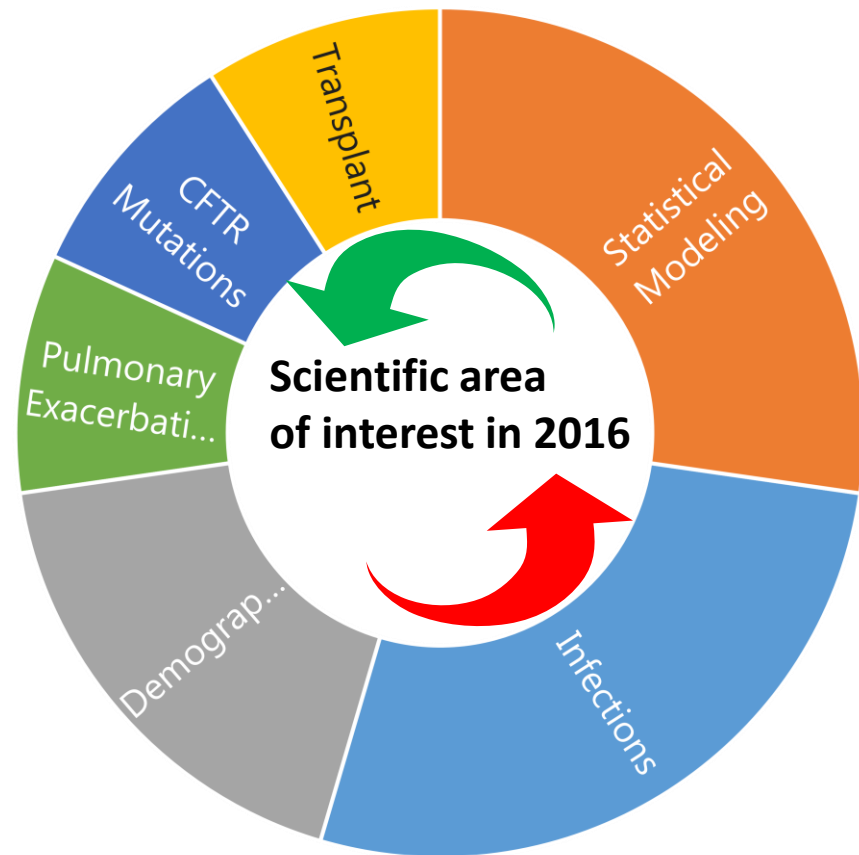
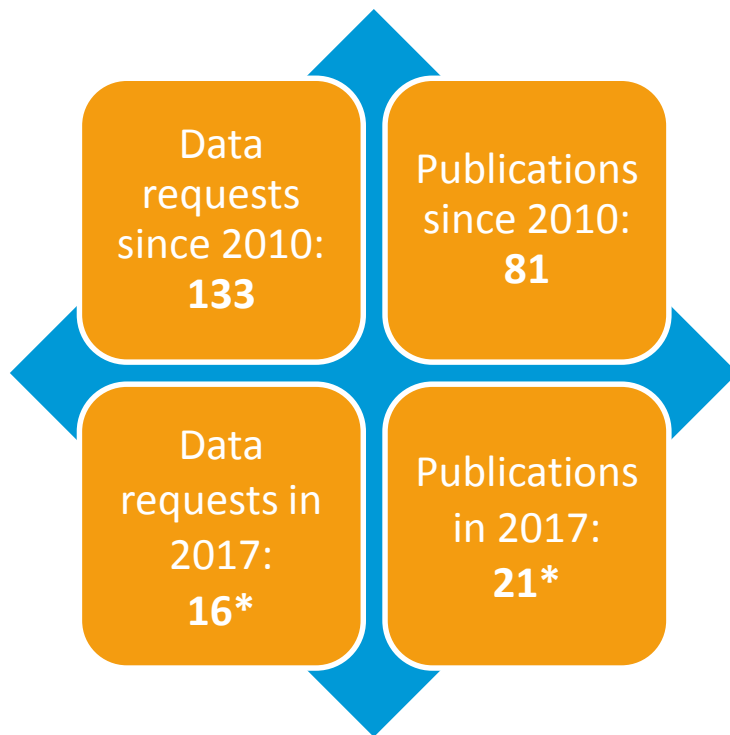
[Author information](#) ► [Copyright and License information](#) ►

US UK Registry Data Comparison



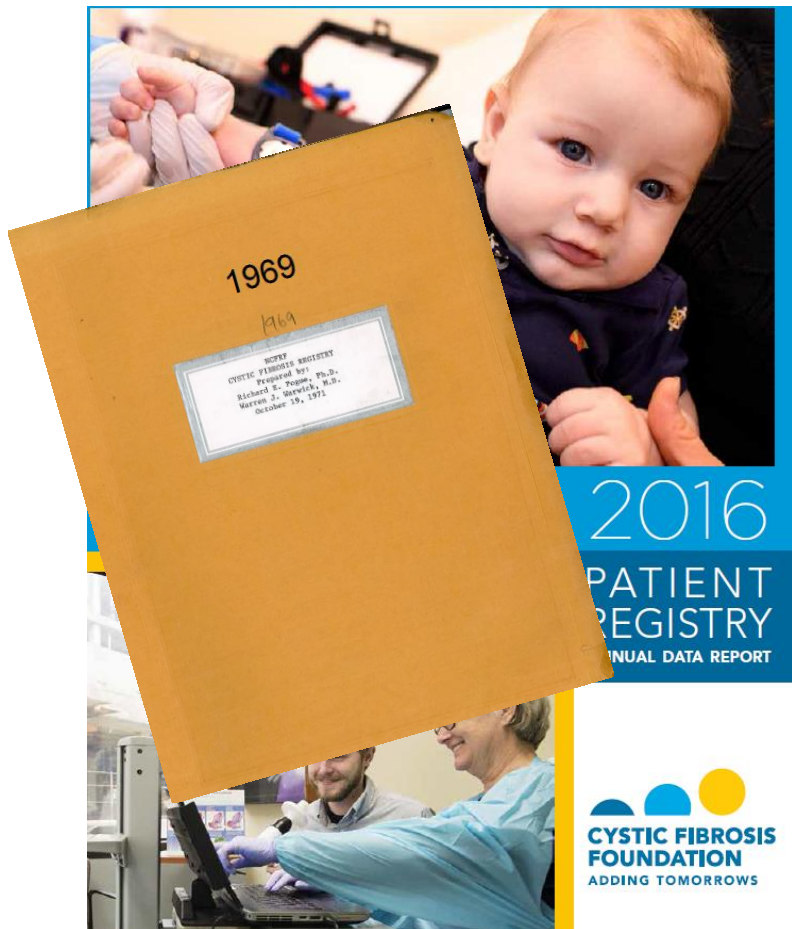
Achieving equal seasonality distribution of recorded encounter records in US and UK CF Registries






* Data for incomplete year


History of Registry Data Reporting



2016 CYSTIC FIBROSIS FOUNDATION PATIENT REGISTRY HIGHLIGHTS



PERCENT OF PEOPLE WITH CF WHO ARE AGED 18 AND OLDER



1996 2006 2016

Improvements in CF care have resulted in more than half of those with CF now aged 18 years and older. Between 1996 and 2016, the number of adults has more than doubled.

Dear CF Community and Friends,

Here are the 2016 Cystic Fibrosis Foundation Patient Registry Highlights. Please use this information to educate families and friends and to engage care providers and policymakers.

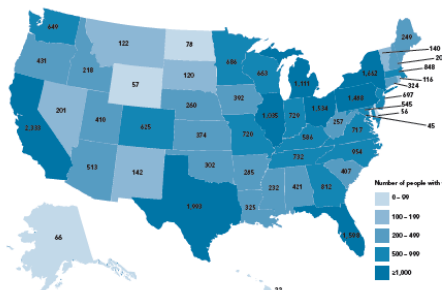
The Registry includes over 25 years of data from the great majority of individuals with CF in the United States. It shows increased survival with adults making up more than 50% of the CF population. We observe steady gains in lung function and nutritional status and decreased presence of harmful lung bacteria. Multiple treatments are commonly used, including the recently approved CFTR modulators.

However, challenges remain including hospitalizations for pulmonary exacerbations, CF-related diabetes, depression, and complex, time-consuming treatment regimens. The CF Foundation has ongoing programs addressing these challenges.

This report reflects dedicated teamwork by CF care center staff alongside people with CF and their families who consent to share their health information. We appreciate your continued commitment to the CF Foundation Patient Registry as an important tool in achieving our mission to cure CF and provide all people with the disease the opportunity to lead full, productive lives.

Sincerely,
Bruce C. Marshall, MD
Senior Vice President
Clinical Affairs

NUMBER OF PEOPLE WITH CF IN THE REGISTRY BY THEIR STATE OF RESIDENCE



In 2016, there were **29,497** people with CF in the Registry, showing a steady increase over time.

Before 2003, all CF registry reports were only available as a printed copy

Center Level Reports

Median FEV1 Percent Predicted for Patients 6 to 17 Years

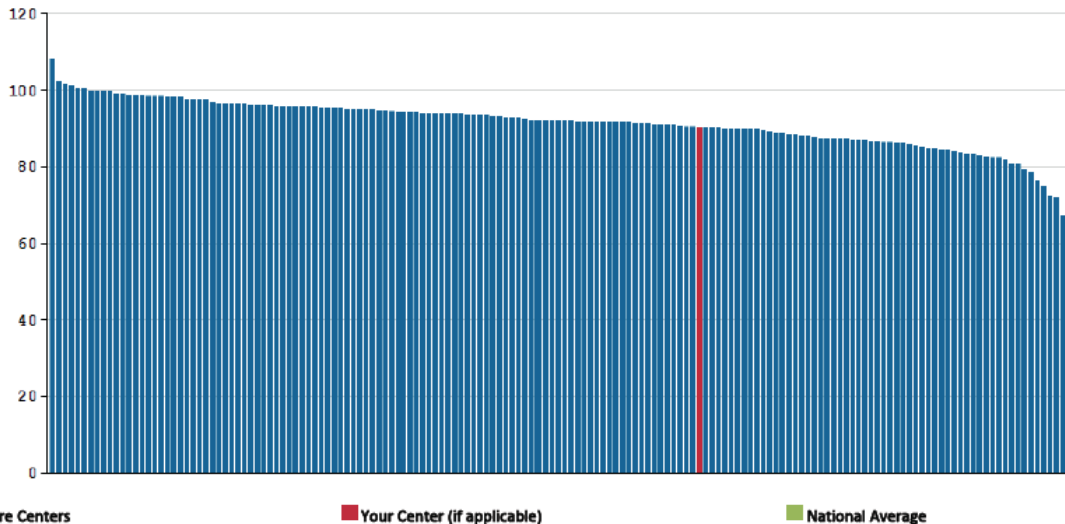
Anonymous Center, 2015 **90.1**

Care Center Network - Median of All Center Values, 2015 92.0

Care Center Network - Average of Top 10 Center Values, 2015 99.9

National Average of All Patients, 2015 92.9

Median FEV1 Percent Predicted for Patients 6 to 17 Years in 2015 ,by Center



Reports provided to each CF Center at the end of Reporting year;

Reports cover almost every important outcome measure ;

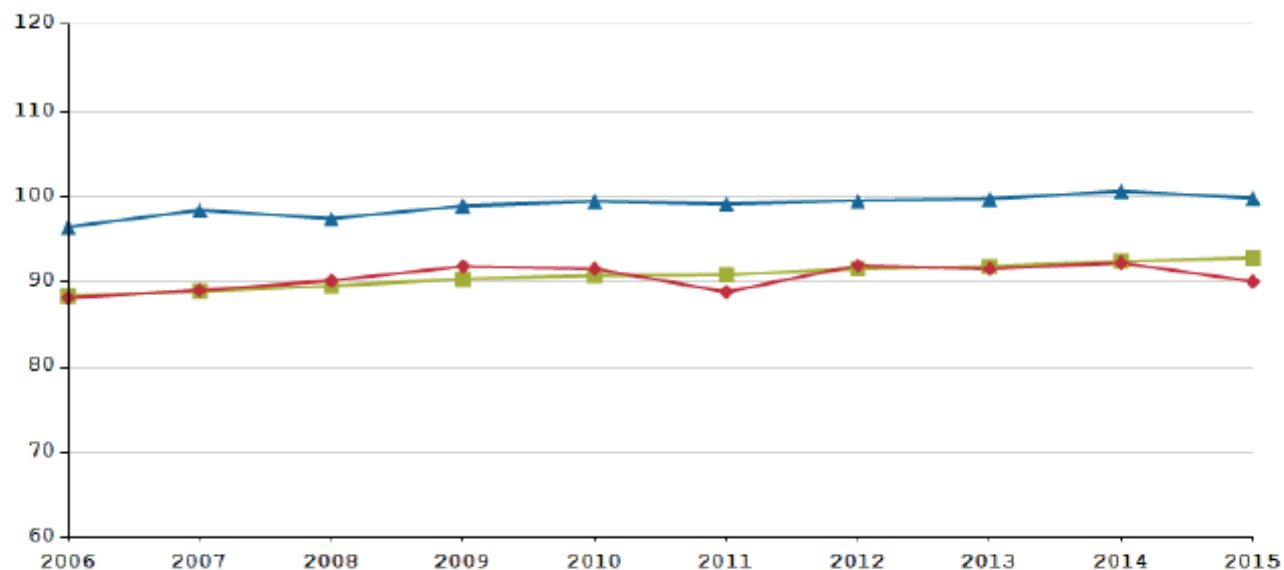
Reports show data longitudinally and cross-sectionally;

Reports allow to compare network-wide results with center-level data;

Reports have over 250 pages of charts and tables.

Median FEV1 Percent Predicted for Patients 6 to 17 Years

Median FEV1 Percent Predicted for Patients 6 to 17 Years, 2006-2015



—▲— Ten Best Performing Centers (if applicable)

—■— National Average

—◆— Your Center (if applicable)

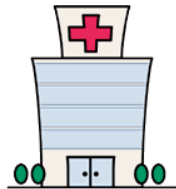
	2006	2007	2008	2009	2010	2011	2012	2013	2014	2015
Your Center	88.2	89.1	90.2	91.9	91.6	88.9	92.0	91.6	92.3	90.1
National Average	88.4	89.0	89.6	90.4	90.8	90.9	91.6	91.9	92.5	92.9
Ten Best Performing Centers	96.5	98.5	97.5	99.0	99.5	99.2	99.6	99.8	100.7	99.9

Bringing Registry Data to Point of Care

January, 2017

~~ONE YEAR~~

24 hours or less



CF Clinic

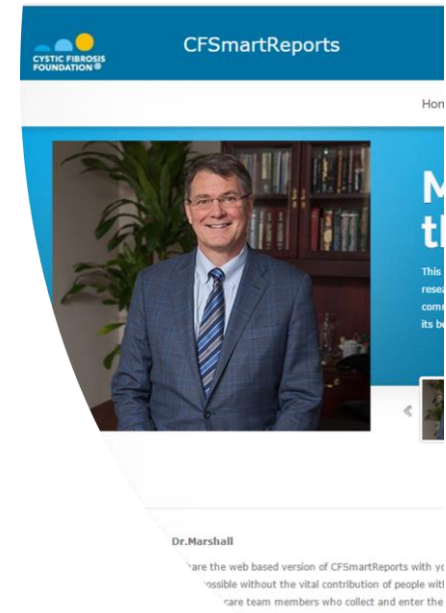
Data
Collection
in PortCF

**CFSmartReports with
patient-level data
deployed!**



CF Registry

Data
Transfer to
CFF and
Cleansing



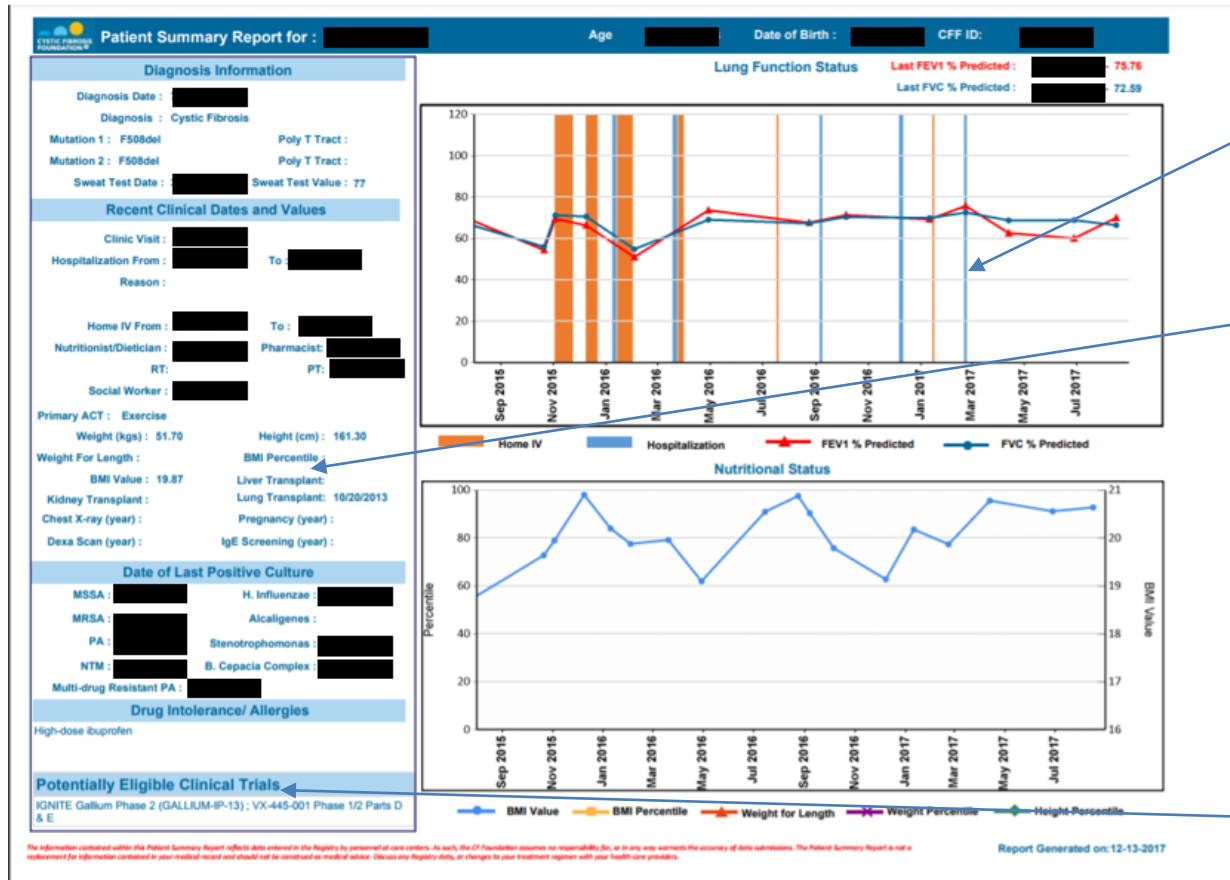
Data
Reporting

Data
Processing

Dr. Marshall

are the web based version of CFSmartReports with y
ossible without the vital contribution of people with
care team members who collect and enter the

Patient Summary Report



CFSmartReports shows Hospitalizations and Home IV events right on FEV1 chart

Microbiology and Vital signs on the same page

Each report may inform Care Team of a patient's clinical trial eligibility

... and much more on other three pages!

Shows patients eligible for clinical trials!

Study: NTM-OB-17 Part A (PREDICT) **Other:** **Status:** Protocol Reviewed
Sponsor: Nick, Jerry **Study Phase:** **Date query deployed:** 8/14/2017
Study Type: Observational **Intervention Type:** **Program Number :** 1
Number of Potentially Eligible Patients: 5

[Solicitations](#) [Recruiting Studies](#) [Referral Studies](#)

Trial Name	Sponsor	Study Phase	Status	Study Type	Intervention Type	
Proteostasis Phase 1 (PTI-808) PART 3		1	Protocol Reviewed	Interventional	Restore CFTR Function	Potentially eligible Patients
NTM-OB-17 Part A (PREDICT)		Other	Protocol Reviewed	Observational		Potentially eligible Patients
CHEC-SC		Other	Protocol Reviewed	Observational		Potentially eligible Patients
AbbVie Creon Phase 4 (M16-111)		4	Protocol Reviewed	Interventional	Nutritional-GI Therapies	Potentially eligible Patients
CURx Phase 2b (CX-FTI-204)		2	Protocol Reviewed	Interventional	Anti-Infective	Potentially eligible Patients
Laurent Phase 2 APPLAUD		2	Protocol Reviewed	Interventional	Anti-Inflammatory	Potentially eligible Patients
Novartis QBW276 Cohort 3		2	Protocol Reviewed	Interventional	Mucociliary Clearance	Potentially eligible Patients
Corbus JBT101-CF-002 Phase 2 PROPOSED		2	Not Reviewed	Interventional	Anti-Inflammatory	Potentially eligible Patients

Queries for 19 clinical trials as of Jan, 2018

List of queries is revisited regularly . New report is usually published in 1-2 weeks after decision to add it

Annual Data Reports	Center Specific Reports	Data Entry Analysis	Population Management Reports
---------------------	-------------------------	---------------------	--------------------------------------

Please choose your Program: *	9001/9001:My Test Pediatric Program ▼
Please choose the Report Type: *	Program Reports ▼

Reports
Patient List
Not seen by a Respiratory Therapist (RT)
Not seen by a Physical Therapist (PT)
Not seen by a Dietitian/Nutritionist (RD)
Not seen by a Social Worker (SW)
Not seen by a Pharmacist (Pharm)
Patients with no Influenza vaccination
Patients taking Enzymes (pancreatic insufficient)
Patients with positive <i>P. aeruginosa</i> (PA) culture
Patients with MDR-PA (multidrug-resistant <i>P. aeruginosa</i>) culture
Patients with positive MRSA (methicillin-resistant <i>S. aureus</i>) culture
Patients with positive <i>Burkholderia</i> culture
Patients age 3 and older with <50% (GLI) FEV1 percent predicted
Patients age < 2 years with <50th Weight-for-Length percentile
Patients age 2-19 years with <50th BMI percentile
Patients age 20+ with BMI under Goal (Females<22, Males<23)

Data Quality Measures vs My Cohort

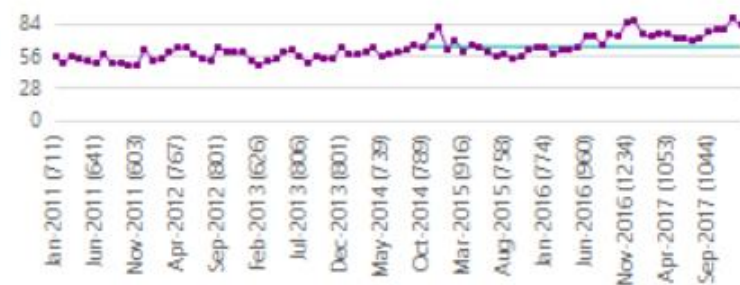
A report of all data quality measures for your program and the appropriate comparison cohort. The comparison cohort includes all programs that joined the CFLN as part of the same onboarding wave but it does not take into account the program type.

Percentage of Clinical Encounters Entered Within 30 days of Encounter Date ↑

Program Name: **6/6 Ann and Robert H. Lurie Children's Hospital of Chicago (Pediatric)**



Program Name: **CFLN (Cohort 1) - All Programs**

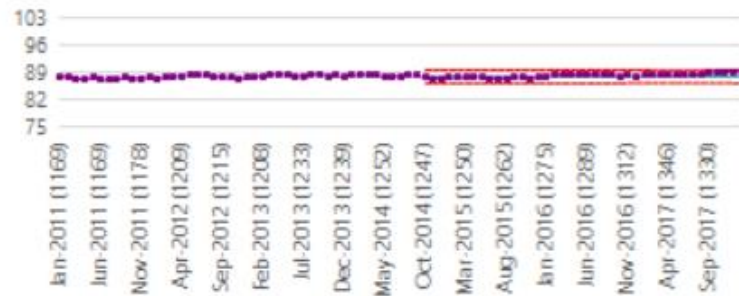


Average FEV1 Percent Predicted for Patients 6 Years and Older ↑

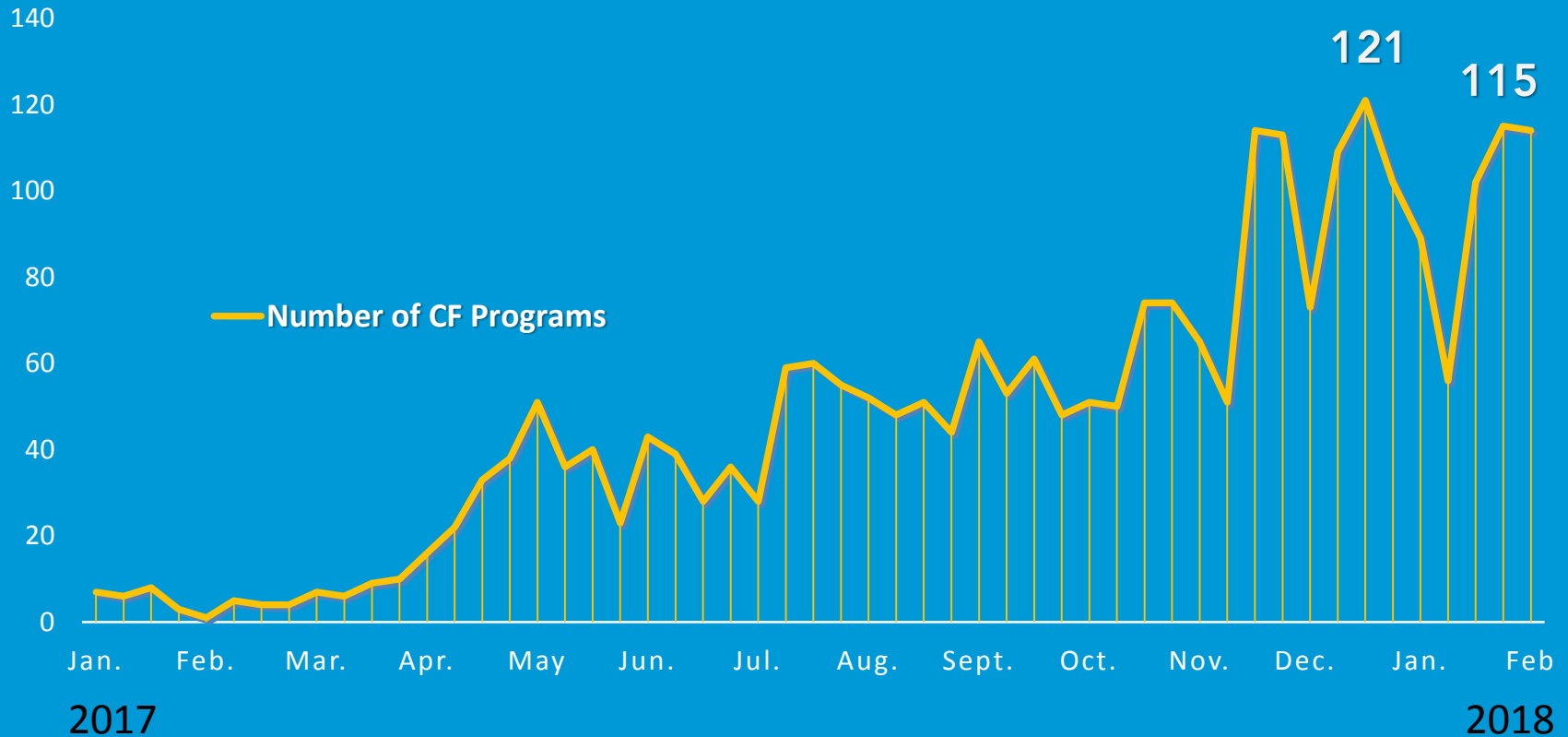
Program Name: **6/6 Ann and Robert H. Lurie Children's Hospital of Chicago (Pediatric)**



Program Name: **CFLN (Cohort 1) - Pediatric Programs**



Uptake of CF SmartReports



Care Teams about CFSmartReports

- 1. Saves them an incredible amount of time to not have to dig through labs, charts, etc... joy in work!**
- 2. Allows them to use their time more valuably to track down referrals and other important info**
- 3. Provides great historical information about each patient, which has really aligned their team around supporting a patient/ family (they project the report on the screen during PVP meetings and have had some great team dialogue about care)**
- 4. Serves as a great data quality check – they have found some missing data variables based on reviewing the reports together**
- 5. Motivates their team to be more responsive to changes in data - e.g. respond to a small change in lung function that they may not have responded to before**
- 6. Highlights which patients are not consented for PortCF and getting them on board so they can benefit from these reports**

Thank you!

Contact: aelbert@cff.org