2016 CYSTIC FIBROSIS FOUNDATION PATIENT REGISTRY HIGHLIGHTS

CYSTIC FIBROSIS FOUNDATION
ADDING TOMORROWS

CF BY THE NUMBERS: 2016

84%
of people with CF in the US are estimated to be in the Registry

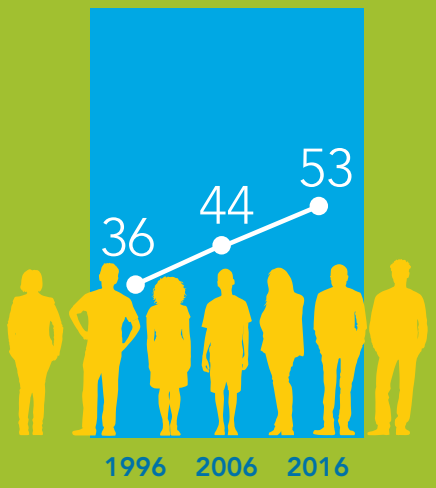
126,615
clinic visits

21,493
hospitalizations

1,478
people in the Registry had a lung transplant

More information on the CF Foundation Patient Registry can be found on CFF.org.

PERCENT OF PEOPLE WITH CF WHO ARE AGED 18 AND OLDER



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.

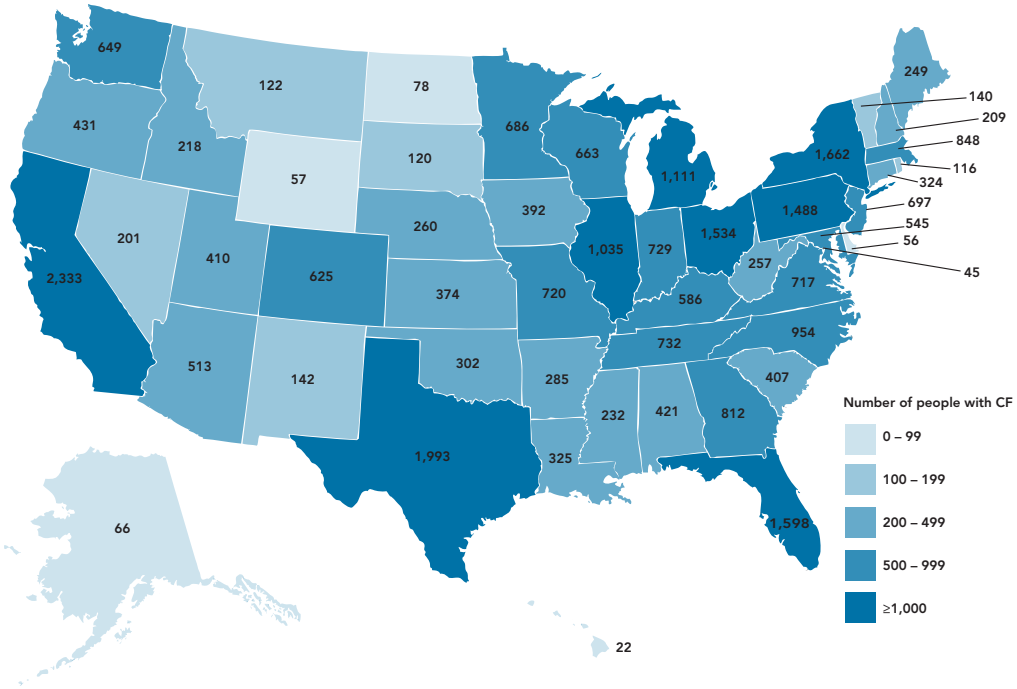
The Registry includes over 25 years of data from the great majority of individuals with CF in the United States. It shows increased survival with adults making up more than 50% of the CF population. We observe steady gains in lung function and nutritional status and decreased presence of harmful lung 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.

DIAGNOSIS FOLLOWING A POSITIVE NEWBORN SCREEN

The great majority of newly diagnosed infants are now detected by newborn screening (NBS). This allows for earlier treatment before onset of signs and symptoms of CF, which has long-term benefits. However, many infants with a positive NBS are found not to have CF after sweat testing and evaluation at a CF care center. Others may have an inconclusive diagnosis (CRMS/CFSPID) and should be followed periodically at a CF care center. This table shows the typical features of infants with CF and CRMS/CFSPID.

	CF	CRMS/CFSPID*
SYMPTOMATIC AT DIAGNOSIS?	YES or NO	NO
SWEAT CHLORIDE (MMOL/L)	60+ — and/or — 2 disease-causing mutations	<60 — and — <2 disease-causing mutations
GENOTYPE		

*The term CRMS (CFTR-related metabolic syndrome) is used in the United States and CFSPID (CF Screen Positive, Inconclusive Diagnosis) is used in other countries. Both terms describe an inconclusive diagnosis following NBS.

MEDIAN BMI PERCENTILE FOR 2-19 YEAR OLDS

2006	2011	2016
47	51	55

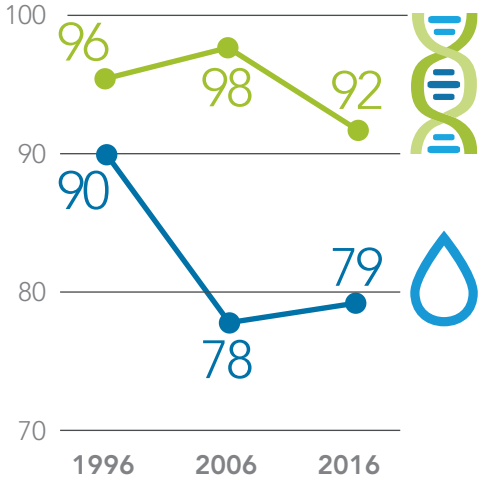
Children and teens with CF need adequate nutrition to grow and thrive, but it is a challenge due to the impact of CF on their digestive systems. The Body Mass Index (BMI) percentile goal is 50 or greater.

PERCENT OF ADULTS MEETING BMI GOALS

2006	2011	2016
40	46	51

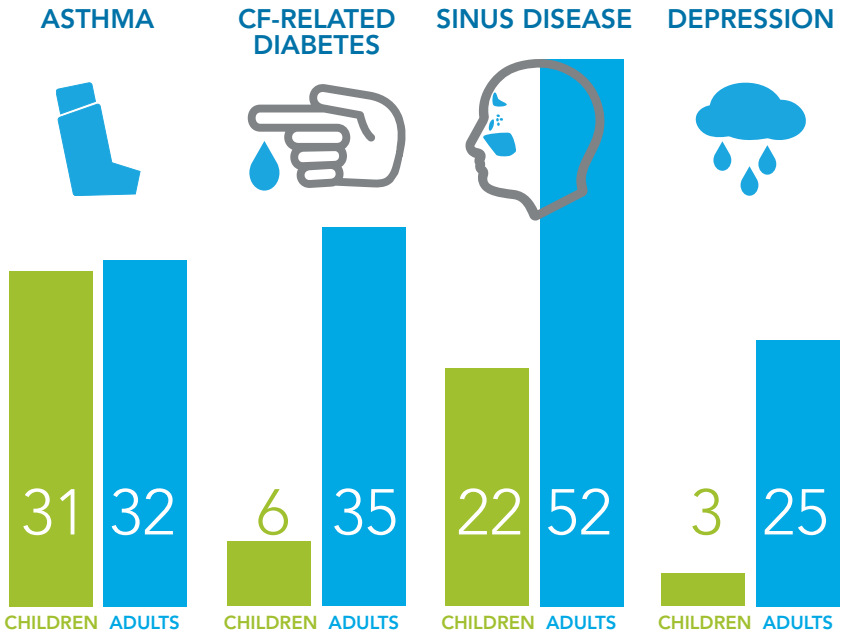
Proper nutrition, which is associated with better lung function, is needed for adults. Over age 20, the BMI goal is 23 for men and 22 for women.

PERCENT OF PEOPLE WITH CF WITH GENOTYPE AND SWEAT TEST



Testing for the genetic mutations that cause CF was first available in the early 1990s. There is a decline in the proportion of people with sweat tests in recent years, but it remains the gold standard for diagnosing CF and an indicator of disease severity and CFTR function.

PERCENT OF PEOPLE WITH CF WHO HAD COMMON COMPLICATIONS IN 2016



CF is a multi-organ system disease.
People with CF can be diagnosed with numerous other complications.

PERCENT OF PEOPLE WITH CF WHO CULTURED POSITIVE IN 2016

People with CF are more vulnerable to lung infections due to the sticky mucus in their lungs. To help prevent this, infection prevention and control guidelines were created for clinics, hospitals, homes, schools, and workplaces.

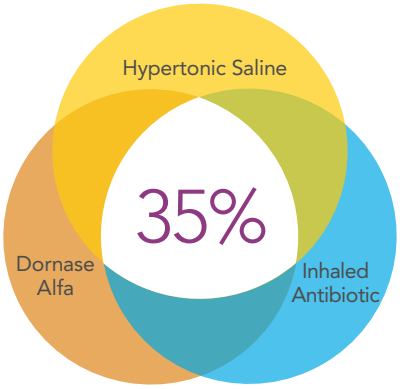
CFTR MODULATORS

7,594
people with CF treated with CFTR modulators.

83%
of people with CF aged 2 or older with a G551D or other gating mutation are on ivacaftor.

In 2012, ivacaftor, the first treatment to target the underlying cause of CF, was approved for people with a G551D mutation and later for other similar gating mutations. In 2015, a second treatment, a combination drug, ivacaftor/lumacaftor, was approved for people with two copies of the F508del mutation.

TREATMENT COMPLEXITY

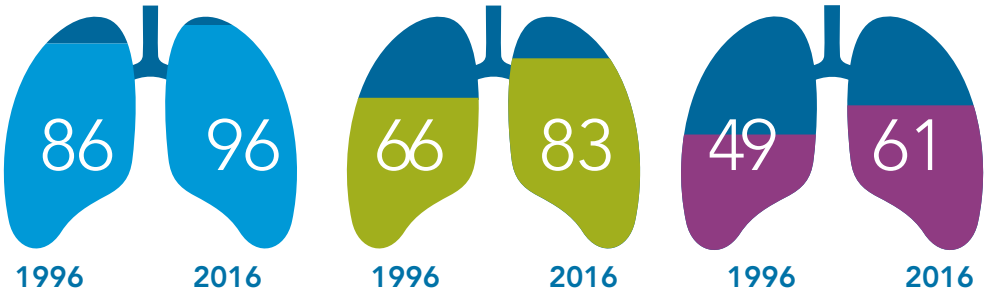


OF PEOPLE WITH CF TAKE THREE OR MORE INHALED MEDICATIONS

Therapies to manage CF symptoms include pills, airway clearance, exercise, inhaled medications, insulin and nutritional supplementation. These therapies help people lead healthier lives, but can require complicated treatment plans that may take 2 – 3 hours per day.

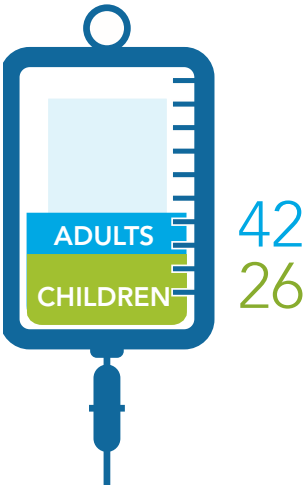
LUNG FUNCTION

MEDIAN FEV_1 PERCENT PREDICTED IN 1996 AND 2016



Lung function is a primary indicator of health and contributes to survival for people with CF.

PERCENT OF PEOPLE WITH CF WITH ONE OR MORE PULMONARY EXACERBATIONS



Pulmonary exacerbations, when lung symptoms worsen, are common in people with CF. Shown above are those treated with intravenous (IV) antibiotics.

INSURANCE

56% of people with CF who are 18-25 years old are on their parents' insurance plan

17% of people with CF who are 18-64 years old are on Medicare

50% of people with CF who are under 10 years old are on Medicaid

CF care can be expensive and includes many out-of-pocket costs for outpatient medical care, hospitalizations and medications. Almost all people with CF have health insurance and many are enrolled in government-funded insurance programs.

LIFE EXPECTANCY AT BIRTH

People with CF continue to enjoy longer and healthier lives. People born between 2012 and 2016, on average, can live to be 43 years of age. Life expectancy estimates do not yet account for the potential benefits of newly available CFTR modulators and other recent clinical care improvements.

CYSTIC FIBROSIS FOUNDATION
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SOURCE OF DATA
Cystic fibrosis patients under care at CF Foundation-accredited care centers in the United States, who consented to have their data entered.

SUGGESTED CITATION
2016 Cystic Fibrosis Foundation Patient Registry Highlights
Bethesda, Maryland