



Cystic Fibrosis Foundation

Patient Registry

Annual Data Report

2002

Suggested citation:

Cystic Fibrosis Foundation,
Patient Registry 2002 Annual Report,
Bethesda, Maryland.

© 2003 Cystic Fibrosis Foundation

Cystic Fibrosis Foundation

6931 Arlington Road
Bethesda, Maryland 20814

toll free (800) FIGHT CF

local (301) 951-4422

internet www.cff.org

e-mail info@cff.org

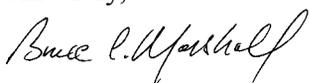
October 1, 2003

The Cystic Fibrosis Foundation's mission is to develop the means to cure and control cystic fibrosis (CF) and to improve the quality of life for those with the disease. The *CF Foundation Patient Registry Annual Data Report* (Patient Registry) shows that CF care is improving. Our goal is to speed up the rate of that improvement. Our main goals can be reached if the whole team works together. This team consists of people with CF, families, physicians, nurses, dietitians, therapists, social workers, plus friends, volunteers and donors who raise money for CF Foundation-supported research. These goals are:

- 1 People with CF and their families are full members of the care team. Communication will be open so everyone can be involved in decisions about care. Care will be respectful of patients' needs, preferences and values.
- 1 Children and teens will have normal growth and good nutrition. Adults' nutrition will be maintained as near to "normal" as possible.
- 1 Everyone with CF will receive the right therapies to keep lung function steady and to decrease the number of respiratory infections or pulmonary exacerbations. These will be diagnosed early and treated quickly.
- 1 People with CF and care teams will work together to eliminate the chances of patients getting respiratory pathogens or germs, particularly *Pseudomonas aeruginosa* and *Burkholderia cepacia*, in the hospital, clinic and home settings.
- 1 People with CF will be closely monitored for complications of CF, mainly CF-related diabetes (CFRD). When someone is diagnosed with CFRD, or any complication of CF, they will be treated quickly.
- 1 Everyone with CF will be able to receive appropriate therapies, treatments and support regardless of race, age, education or insurance coverage.
- 1 Everyone with CF will be supported by their CF team when making decisions about transplantation and end-of-life care.

The pursuit of quality care is the right thing to do and now is the right time to do it. Longevity and quality of life have to be our TOP priorities. Our goals are not just words or ideas, rather that they are real and measurable. Throughout this report we will show data from the Patient Registry that relate to these goals.

Sincerely,



Bruce C. Marshall, M.D.

Director of Clinical Affairs

Cystic Fibrosis Foundation Therapeutics, Inc.

What Is the Cystic Fibrosis Foundation Patient Registry?

More than 30 years ago, the Cystic Fibrosis Foundation started a Patient Registry to track the health of people with cystic fibrosis (CF) across the United States. Today, information about more than 23,000 people who receive care at CF Foundation-accredited care centers is collected and added to the Patient Registry every year. The type of information collected includes state of residence, height, weight, gender, genotype, pulmonary function test results, pancreatic enzymes, length of hospitalizations, home IVs, and complications related to CF.

Why Is the Cystic Fibrosis Foundation Patient Registry Important?

The information collected for the Patient Registry helps find trends and patterns that could lead to better treatments. Because many CF care centers provide care for less than 100 people with CF, it is hard for them to find new trends. However, information in the Patient Registry has helped caregivers see new trends, develop new therapies, and design research studies to help people with CF. The following pages contain information from the Patient Registry.

If You Are New to Cystic Fibrosis

What Is Cystic Fibrosis?

CF is a genetic disease caused by an altered gene. It results in the faulty transport of salt in organs such as the lungs and the pancreas. This leads to thick, sticky mucus that blocks the ducts in these organs, disrupting the normal functions. Many people with CF have a cycle of lung infection and inflammation. This cycle slowly decreases the ability of the lungs to provide oxygen to the body. When the pancreas is affected, it causes problems with digestion and makes it difficult to grow normally and keep a healthy body weight.

Approximately one in 2,500 children in the United States each year is born with CF. It is found in all racial and ethnic groups, however it is more common among Caucasians. An estimated 30,000 people in the United States have the disease.

What Is the Cystic Fibrosis Foundation?

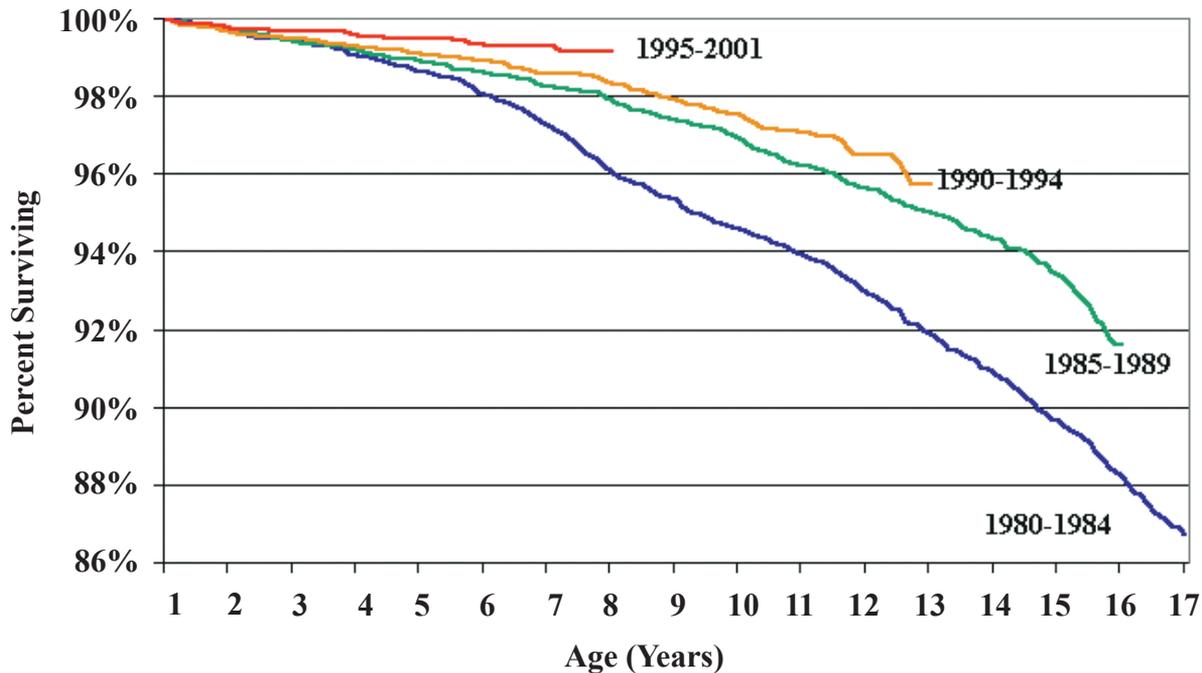
The CF Foundation was created in 1955 by a dedicated group of parents who had children with CF. They had a clear mission — to develop the means to cure and control CF and to improve the quality of life for those with the disease.

To help with this mission, the CF Foundation started a network of more than 115 accredited care centers across the United States to care for people with CF. The CF Foundation provides grants to these centers to help them care for people with CF. The CF Foundation also provides grants to researchers working to find a cure and to control the disease, by discovering and developing new drugs.

Improving Survival

Due to the special care at CF Foundation-accredited care centers, the predicted survival of people with CF has steadily improved. When the CF Foundation was created in 1955, few children with CF lived to school age. Today, the predicted survival extends into the early 30s. Continued improvement in survival depends, in part, on gathering and using data from people with CF across the United States.

Survival from Age One, by Year of Birth



Actual survival of patients in the registry has improved since 1980. Of people with CF born between 1980 and 1984 (the dark blue line), 93 percent were alive at age 12. For children born between 1990 and 1994 (the orange line), 96.5 percent were alive at age 12. Children born between 1995 and 2001 (the red line), are doing even better.

Goal: People with CF and their families are full members of the care team. Communication will be open so everyone can be involved in decisions about care. Care will be respectful of patients' needs, preferences and values.

Before, reports from the Patient Registry data could only be seen once a year. Now, the Patient Registry has instant reports about individuals with CF. The CF Foundation designed reports to help communication in the care team about some of the important care issues of the disease. These reports are available from your CF Foundation-accredited care center. Following is an example of one of the reports, the "Patient Summary Report."

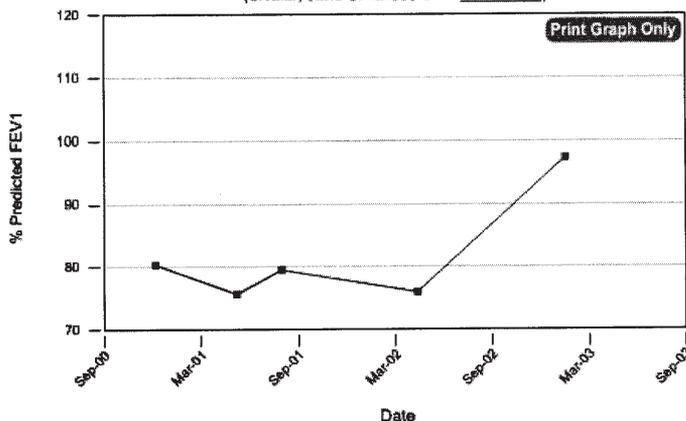
Cystic Fibrosis Foundation Patient Registry: Annual Data Report 2002

VISIT DATE: _____ **Name:** Sivalar, Jane
Last Hospitalization: None **Date of Birth:** 03/25/1992
Last HomeIV Therapy: None **Genotype:** No Date Not Identified/Not Identified
Last Clinical Visit: 1/23/2003

Culture Results
Last Culture: Staphylococcus aureus, Streptococcus Pneumoniae, : 1/23/2003
Last Positive: **B. cepacia** **MRSA** **PA** **MDR-PA+**
 2/15/1997 None 8/22/2002 None

PFT Trend

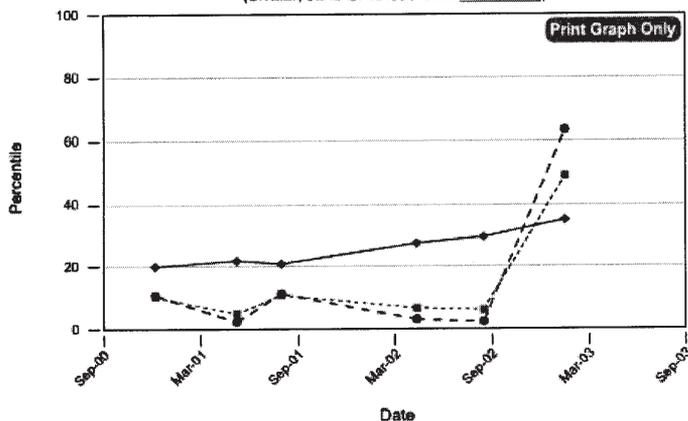
(Sivalar, Jane CPID:999 Date: _____)



FEV1
 Current Value _____

Nutritional Trend

(Sivalar, Jane CPID:999 Date: _____)



Height %ile Weight %ile BMI %ile (ages > 2 yrs)
 Current Value _____ Current Value _____ Current Value _____

Complications

ACTIVE

RESOLVED

None

● PANCREATIC STEATORRHEA

Routine Evaluations

Last PFT: 1/23/2003
 Last CXR: None
 Last SW Visit: 1/23/2003

Last Dietary Visit: 1/23/2003
 Last LFT: 6/1/2002
 Glucose Screening: 6/1/2002
 Creatinine: 4/18/2002

+Prior to 2003, multi-drug resistant *Pseudomonas Aeruginosa* (MDR-PA) status cannot be determined based on registry data.

Comments

Guidelines for CF Care

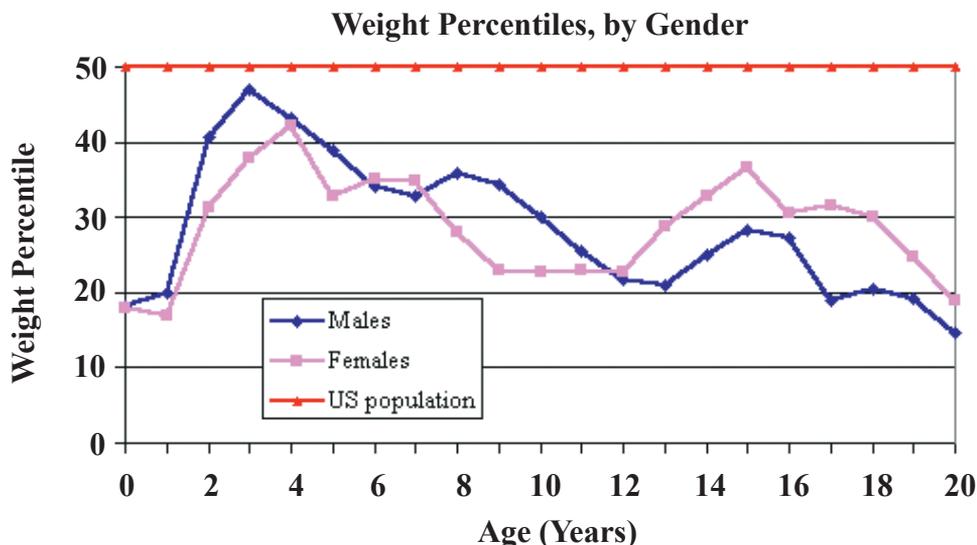
Since the early 1990s, the CF Foundation has gathered experts in CF care to develop guidelines for the care of people with CF of all ages. These guidelines have helped CF care teams improve the quality of life for people with CF. Below are some of these recommendations and what the CF Foundation has found in the Patient Registry.

Guidelines for CF Care	Children Who Meet Guidelines (%)	Adults Who Meet Guidelines (%)
Outpatient Visits — 4 Per Year	66.6	54.7
Pulmonary Function Tests — (PFT) 2 or More Per Year	83.9	75.7
Respiratory Cultures — At Least 1 Per Year	95.8	92.0
Creatinine Level — Every Year	81.0	81.3
Glucose — Every Year if >13 Years	80.9	80.4
Liver Enzymes — Every Year	82.1	79.6

Maintaining Normal Nutrition

Goal: Children and teens will have normal growth and good nutrition. Adults' nutrition will be maintained as near to "normal" as possible.

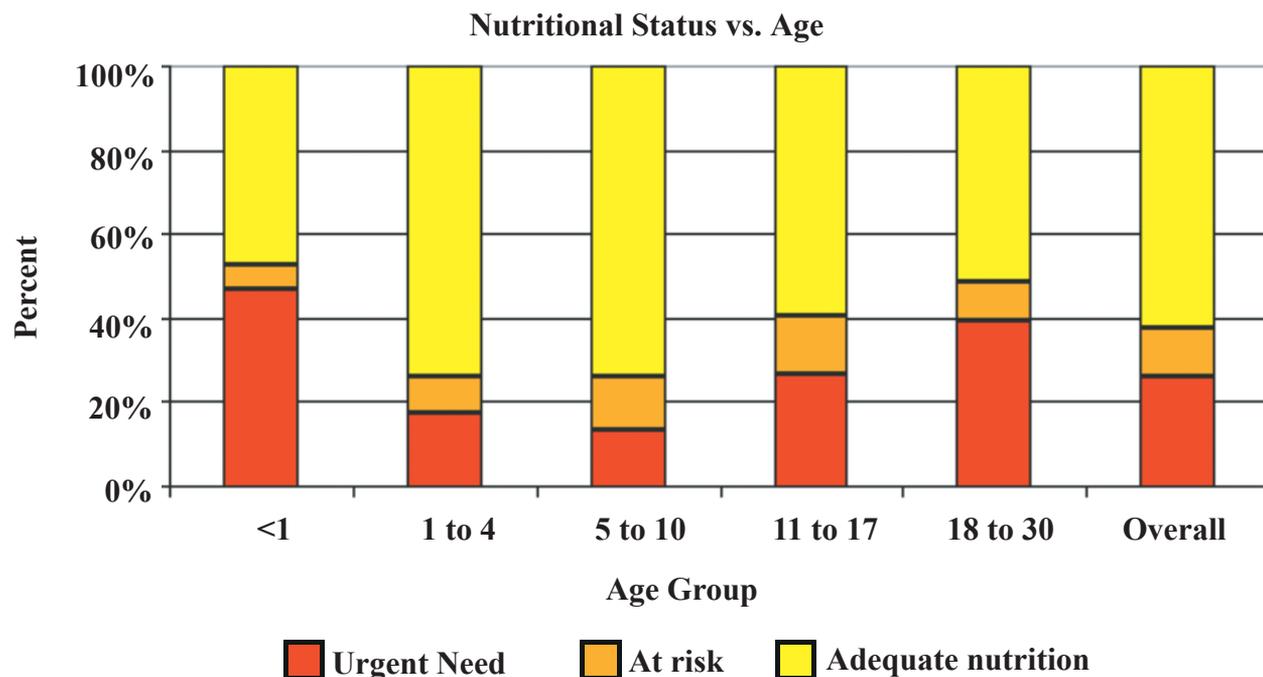
Steady progress toward this goal has been made, but work remains to be done. In 2001, the CF Foundation gathered a group CF care experts to update the guidelines for treating and managing the nutrition of children with CF. The Patient Registry shows a strong link between nutrition and survival. Therefore, dietitians are now key members of each CF care team to help reach the goal of good nutrition for everyone with CF.



Cystic Fibrosis Foundation Patient Registry: Annual Data Report 2002

Today, because of the work of the CF Foundation-accredited care centers to improve nutrition, people with CF are closer to the average population in size. Still, people with CF are usually born smaller than those without CF. CF causes many people to have problems absorbing nutrients and gaining weight. In the previous graph, the general United States population is at the 50th percentile for weight in all ages. In 2002, people with CF, as a group, were always below the middle of the general U.S. population. This shows how hard it is for people with CF to maintain good nutrition.

Once diagnosed and treated, children with CF reach their highest weight percentile around four years of age, but often decline after that. Caregivers need to better understand why children lose ground through childhood, then work to slow or stop that trend. Nutritional supplements are an important way to improve nutrition and increase calories as children grow. Good nutrition can help keep lungs healthy. The problem of poor nutrition and weight continues into adulthood. In people with CF between 18 and 30 years of age, 39.4 percent are in urgent need of improved nutrition, another nine percent are at risk.



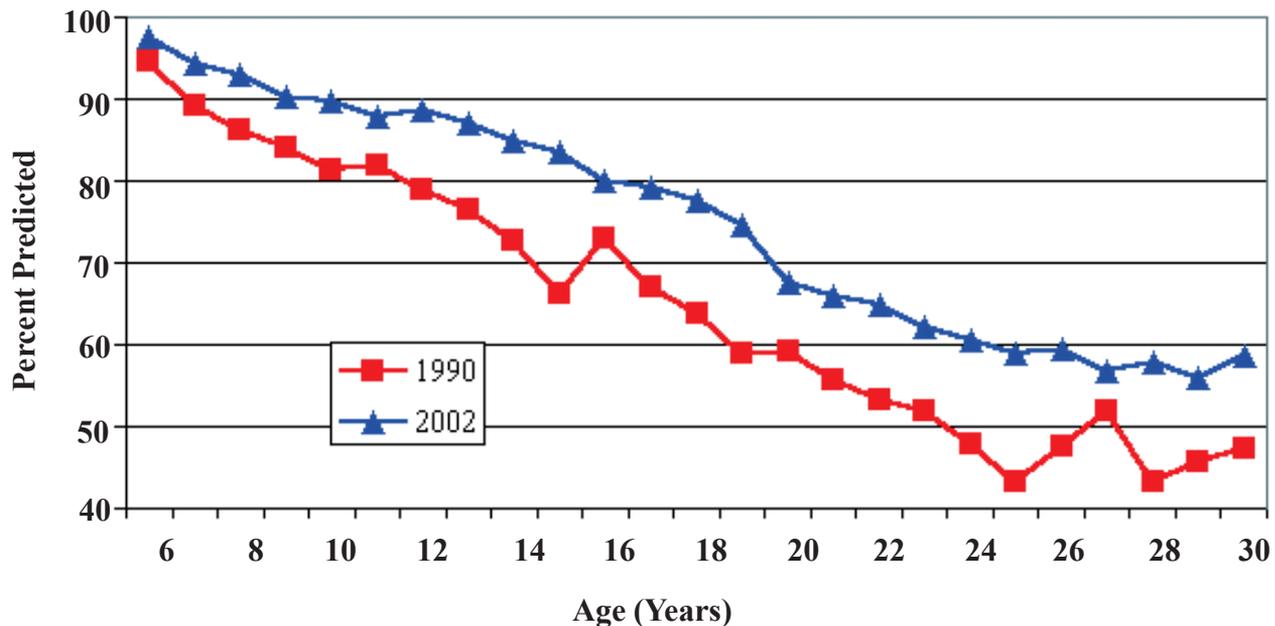
To learn more about CF and nutrition, ask your CF care team for the fact sheets about nutrition. These fact sheets can also be found on the CF Foundation's Web site at www.cff.org.

Lung Function

Goal: Everyone with CF will receive the right therapies to keep lung function steady and to decrease the number of respiratory infections or pulmonary exacerbations. These will be diagnosed early and treated quickly.

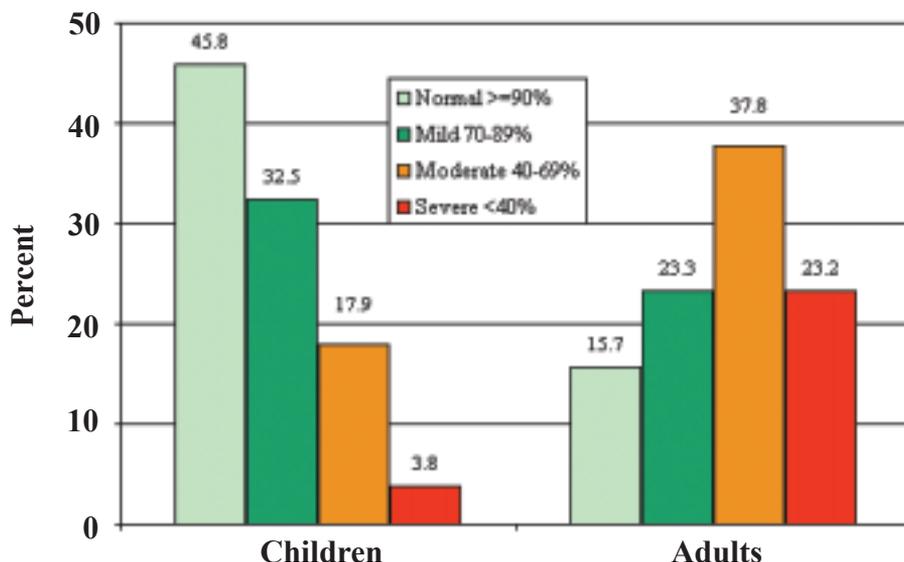
Everyone with CF starts with healthy lungs at birth. Lung function is measured by FEV₁, or Forced Expiratory Volume over one second, which is shown as percent predicted based on a healthy, non-smoking, group of people. It is usually near normal or just under 100 percent when first measured around six years of age. As seen below, lung function then declines at about two percent per year. However, since 1990, lung function has improved five to 10 percentage points for all ages from six to 30 years.

Median Percent Predicted FEV₁ vs. Age, 1990 and 2002



The next graph shows the percentage of children and adults and the severity of lung disease in each group. The lower a person's FEV₁, the more severe the lung disease. An FEV₁ greater than or equal to 90 percent is normal. If the FEV₁ is between 70 and 89 percent, the person has mild lung disease. An FEV₁ between 40 and 69 percent, means the person has moderate lung disease. If the FEV₁ is less than 40 percent, this means severe lung disease is present. The graph shows that adults have more severe lung disease, but many have normal or mild lung disease. Therapies like Pulmozyme[®], antibiotics like TOBI[®] and better nutrition have all worked to improve FEV₁. Airway clearance and exercise also lead to better lung function.

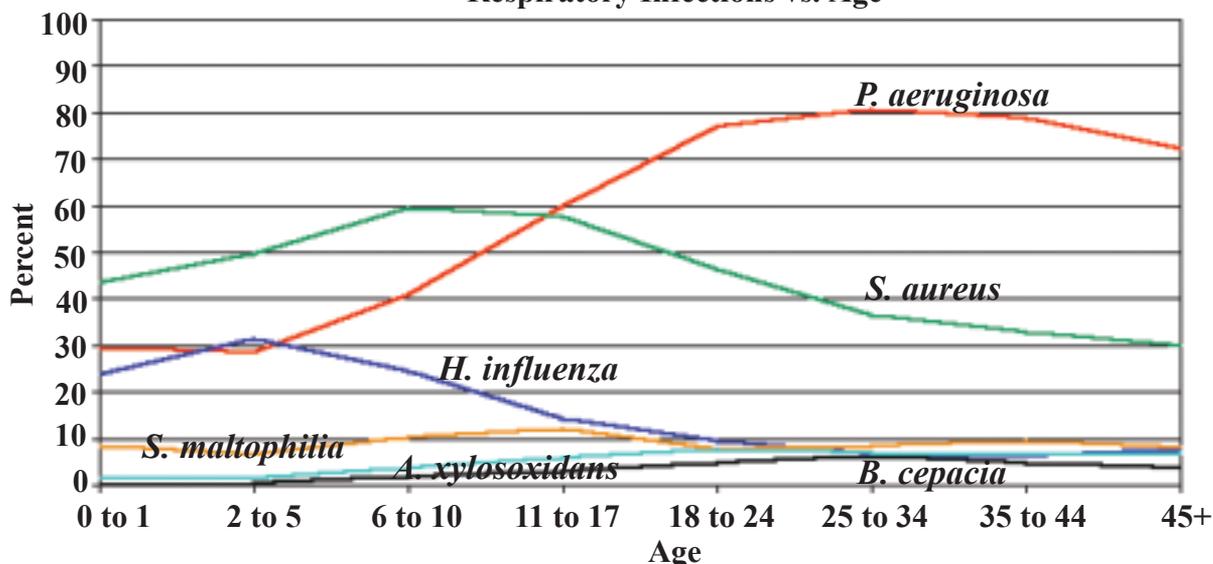
Respiratory Severity for Children and Adults — FEV₁ Percent Predicted



Goal: People with CF and their care teams will work together to eliminate the chances of patients getting respiratory pathogens or germs, particularly *P. aeruginosa* and *B. cepacia*, in the hospital, clinic and home settings.

Repeated respiratory infections or pulmonary exacerbations are a main concern for people with CF. It is the cycle of infection and inflammation that damages the lungs. This damage causes lung function (FEV₁) to decline. When the lungs are damaged, pulmonary exacerbations happen more often. The next graph shows what germs can cause lung infections in different age groups. To learn more about preventing respiratory infections, ask your CF care team. Information about stopping the spread of germs is also on the CF Foundation’s Web site at www.cff.org.

Respiratory Infections vs. Age



Overall Percentage in 2002:

- *P. aeruginosa* 57.8%
- *S. aureus* 49.7%
- *H. influenza* 16.3%
- *S. maltophilia* 9.4%
- *B. cepacia* 3.1%
- *A. xylosoxidans* 5.2%

Complications of CF

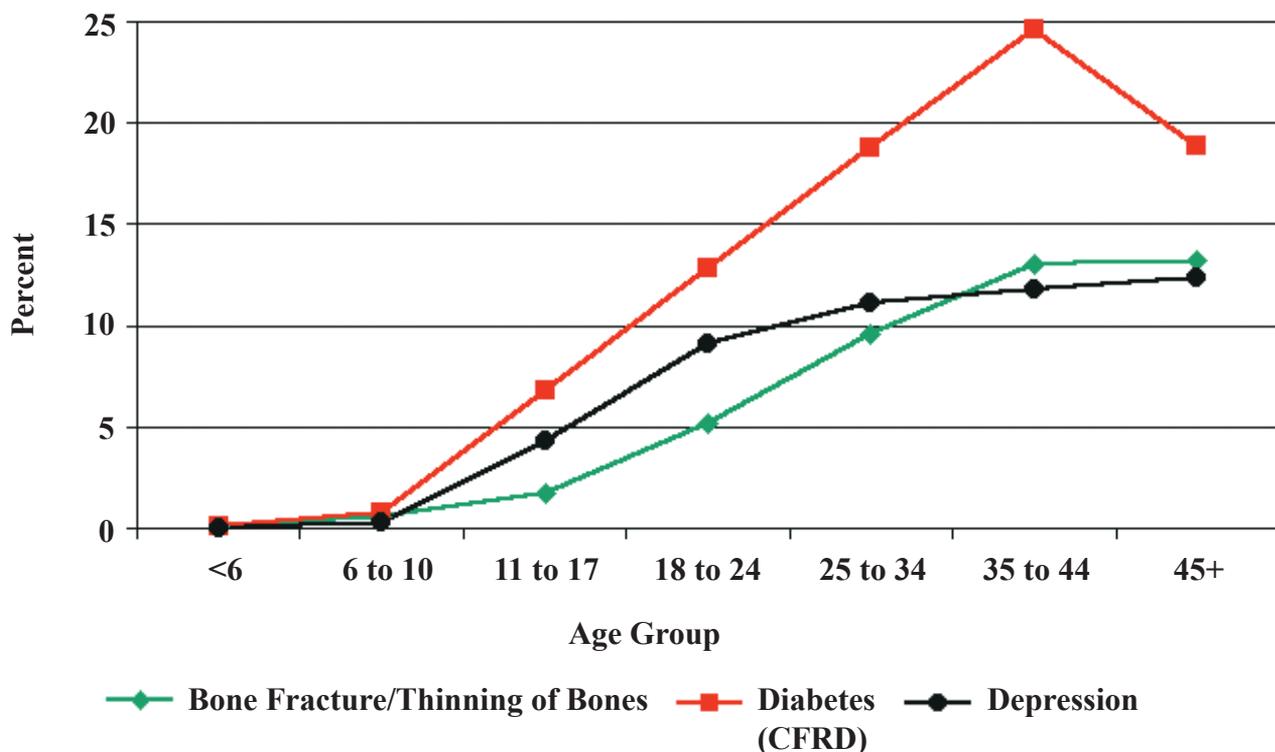
Goal: People with CF will be closely monitored for complications of CF, mainly CF-related diabetes (CFRD). When someone is diagnosed with CFRD, or any complication of CF, they will be treated quickly.

In the early 1990s, data in the Patient Registry showed an increase in the number of teens and adults with diabetes and CF. Based on these data, the CF Foundation funded CF and diabetes research. CFRD was found to be different from diabetes in people without CF. The CF Foundation then brought together experts in CF and diabetes to develop guidelines for this problem. The guidelines outline how to detect and treat CFRD. Anyone with CF, 14 years of age or older, should be screened every year for CFRD. The Patient Registry data suggests that early detection and better treatment of CFRD leads to better nutrition and healthier lungs.

The Patient Registry shows trends in other complications of CF as well. The importance of good nutrition and healthy bones is often in the news. Data from the Patient Registry shows that about 8.5 percent of adults with CF had signs of poor bone health in 2002. This led the CF Foundation to gather experts in bone health and CF to develop guidelines to screen and treat bone disease in people with this disease. These guidelines recommend the best way to keep the bones of people with CF healthy.

Another finding from the Patient Registry is that more than 10 percent of adults with CF have symptoms of depression. This is a common complication of many chronic diseases. The Patient Registry shows that people with CF, families and caregivers need to be aware of this complication. People often respond well to treatment for depression.

Common Complications vs. Age



Access to Care

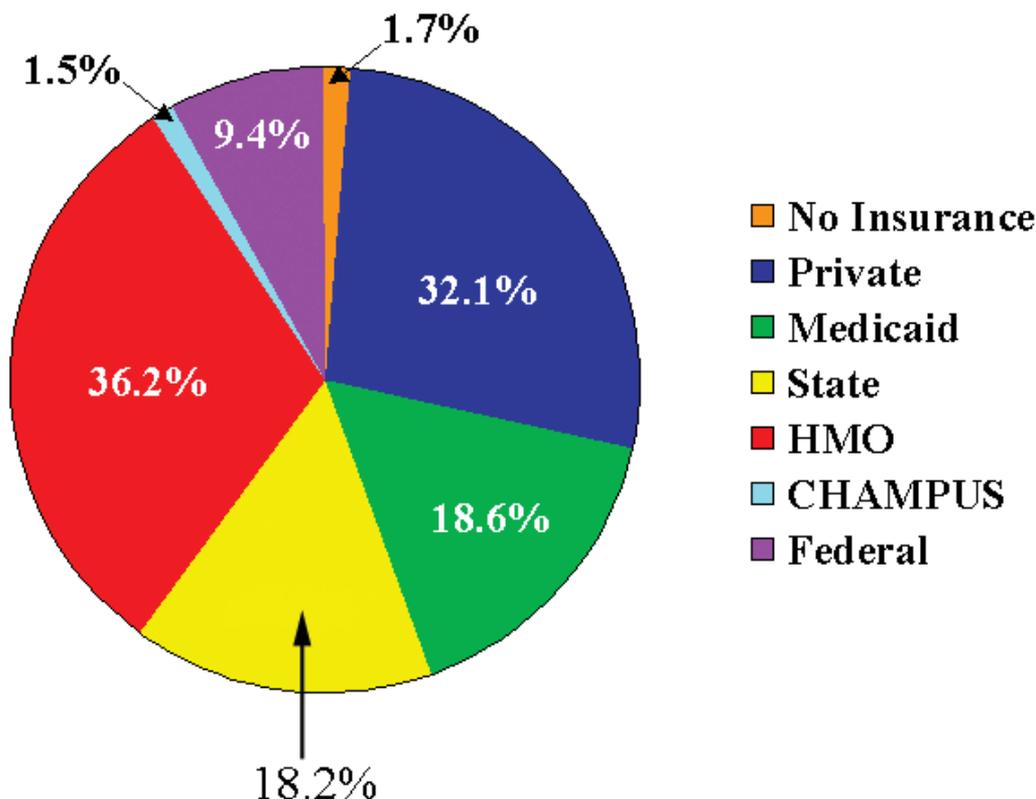
Goal: Everyone with CF will be able to receive appropriate therapies, treatments and support regardless of race, age, education or insurance coverage.

Patient Registry data suggest that people with CF who live in households with lower incomes have poorer lung function and have worse nutrition than others with CF. This pattern of poor health in lower income households is common with other chronic diseases. The CF Foundation is working to find out why this happens in CF and how to change it. The CF Foundation works hard to keep proven CF therapies available to everyone. For example, data from the Patient Registry shows that people with CF, no matter their income, have therapies like Pulmozyme® and TOBI® available to them.

Anyone with CF can get therapies, treatments and support at a CF Foundation-accredited care center. To find the center nearest you, visit the CF Foundation's Web site at www.cff.org.

Learn more about race and age of the people in the Patient Registry by turning to page 12, "A Summary of the 2002 Data."

Insurance Coverage for Adults With CF

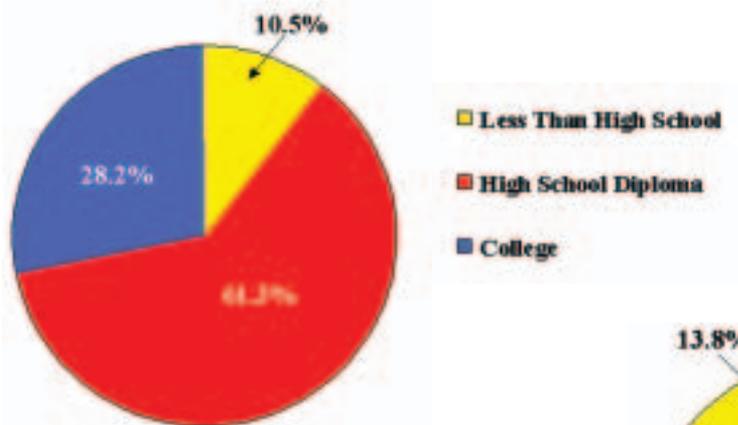


Adults With CF

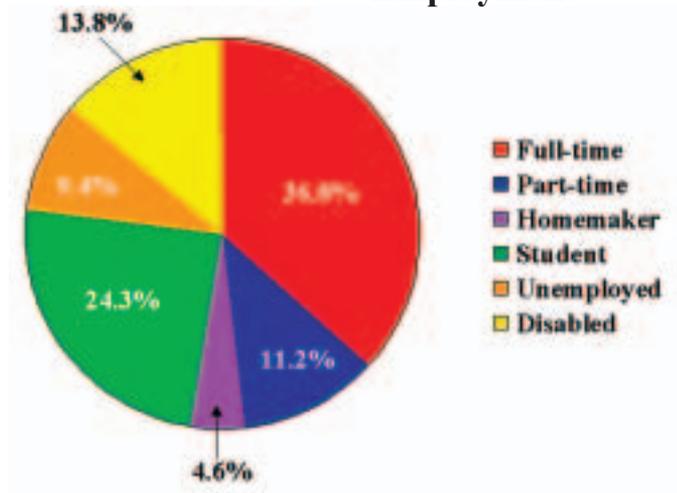
In 1990, about 30 percent of people in the Patient Registry were over the age of 18. The number of adults will continue to rise. Because of this, the CF Foundation brought together experts in CF and adult care to develop guidelines for the care of adults with CF. CF care teams also recognize the importance of helping teens transition from depending on their parents or another adult for healthcare decisions, to taking charge of their own healthcare. In 2002, 40.2 percent of people with CF in the Patient Registry were adults.

Data from the Patient Registry show some interesting facts about adults with CF. As you can see, they lead busy lives while at the same time dealing with CF.

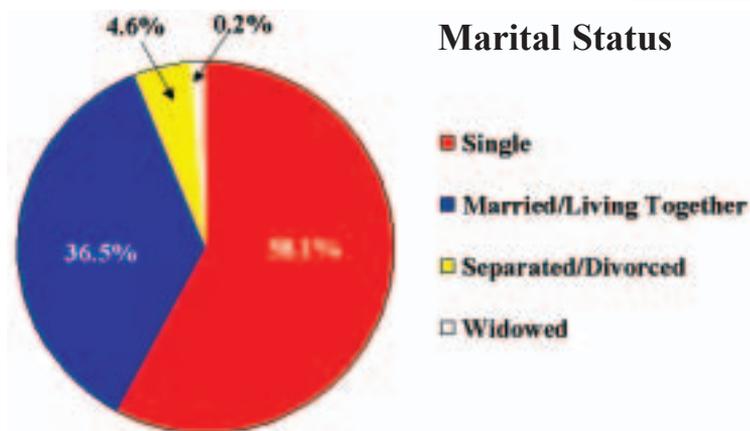
Education



Employment



Marital Status



CF and Pregnancy

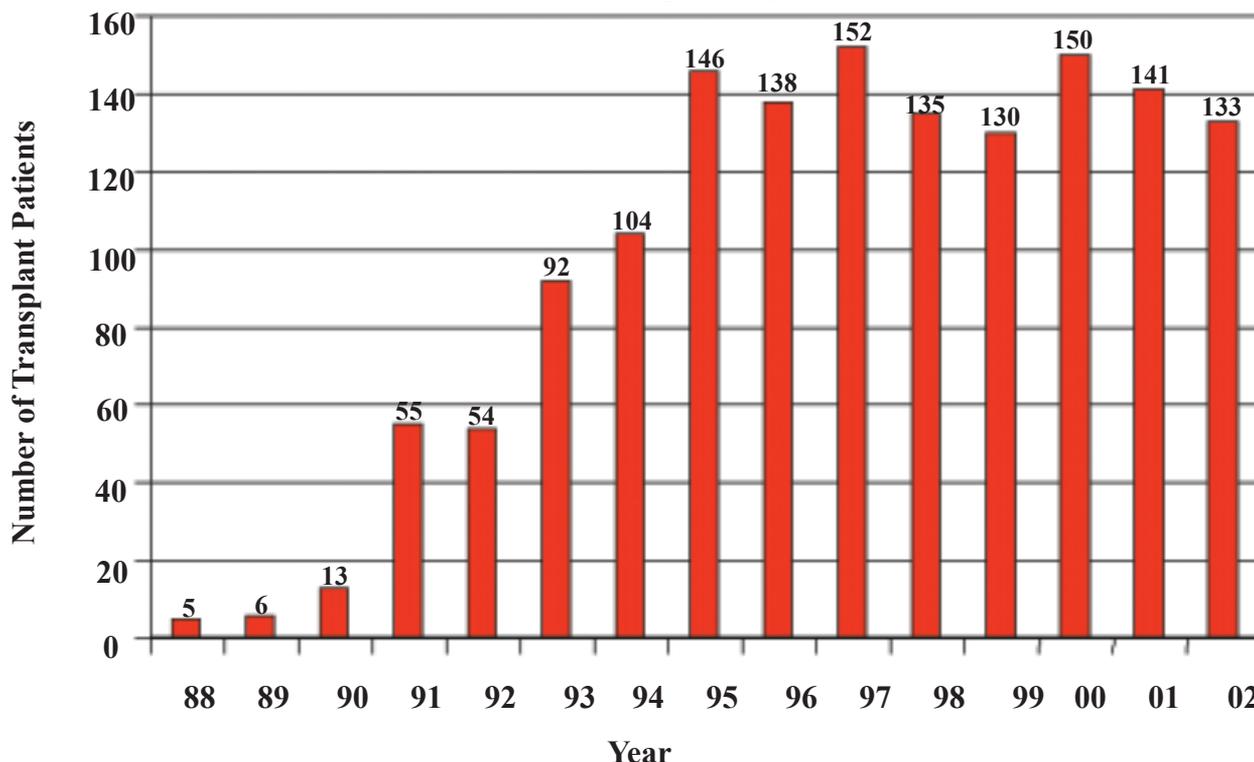
As more females with CF become adults, many wish to have children. In the 1980s, it was thought to be too risky for a woman with CF to get pregnant. Now, thanks to improvements in survival and research done with the Patient Registry, CF care teams can give better advice about the risks of pregnancy. In the year 2002, the Patient Registry reported that 185 women with CF were pregnant.

Transplantation and End-of-Life-Care

Goal: Everyone with CF will be supported by their CF team when making decisions about transplantation and end-of-life care.

Loss of lung function often leads people with CF to think about a lung transplant. Unfortunately, a lung transplant is very risky, the supply of good lungs is limited, and the evaluation to decide if a person can have a transplant takes time. It is important to understand who can benefit most, and when is the best time for a transplant. More than 1,400 people with CF have received lung transplants since 1988. The Patient Registry data has been used to help identify who is most likely to benefit from a transplant. To learn more about lung transplants, visit the CF Foundation's Web site at www.cff.org.

Number of Patients With Lung Transplants 1988 to 2002



To locate a CF Foundation-accredited care center near you, visit the CF Foundation's Web site at www.cff.org.

**Who Are the CF Patients in the Patient Registry —
A Summary of the 2002 Data.**

CF patients (number)	23,105	FEV ₁ % predicted (mean)	74.3
Newly diagnosed patients in 2002 (number)	972	Patients with weight <5 th percentile* (%)	17.3
Age at diagnosis (median)	6 months	Patients with height <5 th percentile* (%)	15.8
Age range	0 to 74 years	Respiratory cultures positive for (%)	
Total number of deaths	421	<i>P. aeruginosa</i>	57.8
Predicted survival age	31.6 years	<i>B. cepacia</i>	3.1
Patients 18 years and older (%)	40.2	<i>S. aureus</i>	49.7
Race/Ethnicity (%)		<i>S. maltophilia</i>	9.4
Caucasian	94.6	Complications (%)	
Hispanic (black or white)	6.4	Diabetes (CFRD)/glucose intolerance	12.0
African American	3.7	Bone disease (patients ≥ 18 years)	9.0
Males (%)	52.8	Liver disease	6.1
Genotyped (%)	81.4	Nasal polyps requiring surgery	3.4
Insurance Coverage (Adults) (%)		Transplants (numbers)	
None	1.7	Lung:	
State/Medicaid	36.8	Bilateral	126
Private/Champus	33.6	Heart-lung	1
HMO	36.2	Lobar-cadaveric	10
Home therapy (%)		Lobar-living related donor	9
IV antibiotics	20.9	Lobar-unrelated donor	1
Oxygen	6.4	Liver:	14
Supplemental feeding – tube	8.7	Therapies (% using medications at least once)	
oral only	31.2	TOBI®	46.5
Taking pancreatic enzyme supplements (%)	92.2	Pulmozyme®	56.3
Clinical trial participation (number)	1,444	Ibuprofen	3.8
		Ursodeoxycholic acid	7.0
		Total pregnancies among women aged 13 to 45 (number)	185
		Live births (per 100 women age 13 to 45)	1.9

*The Centers for Disease Control and Prevention have issued new growth charts – updated weight and height reference tables – for children up to age 20. For more information see www.cdc.gov/growthcharts.

Number of Patients by State in the CF Patient Registry

<u>State</u>	<u>Number</u>	<u>Percent</u>	<u>State</u>	<u>Number</u>	<u>Percent</u>
Alabama	352	1.5	Montana	96	0.4
Alaska	55	0.2	Nebraska	200	0.9
Arizona	369	1.6	Nevada	126	0.6
Arkansas	220	1.0	New Hampshire	181	0.8
California	1,851	8.1	New Jersey	534	2.3
Colorado	441	1.9	New Mexico	118	0.5
Connecticut	289	1.3	New York	1,458	6.3
Delaware	64	0.3	North Carolina	704	3.1
District of Columbia	21	0.1	North Dakota	55	0.2
Florida	1,101	4.8	Ohio	1,316	5.7
Georgia	642	2.8	Oklahoma	231	1.0
Hawaii	12	0.1	Oregon	317	1.4
Idaho	131	0.6	Pennsylvania	1,271	5.6
Illinois	957	4.1	Puerto Rico	46	0.2
Indiana	549	2.4	Rhode Island	106	0.5
Iowa	292	1.3	South Carolina	276	1.2
Kansas	271	1.2	South Dakota	86	0.4
Kentucky	399	1.8	Tennessee	413	1.8
Louisiana	261	1.1	Texas	1,362	6.0
Maine	198	0.9	Utah	220	1.0
Maryland	445	2.0	Vermont	127	0.6
Massachusetts	847	3.7	Virginia	636	2.8
Michigan	835	3.7	Washington	497	2.2
Minnesota	505	2.2	West Virginia	164	0.7
Mississippi	219	1.0	Wisconsin	569	2.5
Missouri	565	2.5	Wyoming	45	0.2
			Foreign	49	0.2

