Cystic Fibrosis Foundation

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Cystic Fibrosis Foundation Patient Registry Annual Data Report 2004

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October 1, 2005

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, but we want to go faster. We are supporting our care centers in this effort by using the following strategies:

- *Developing and sustaining leadership for improvement work.* We have trained more than 30 care centers on state-of-the-art quality improvement (QI) tools and process and plan to train many more.
- *Sharing QI tools and approaches with all care centers.* Key resources are now available to all care centers on Port CF, the Web-based Patient Registry. We are encouraging communication, sharing and teamwork between centers to achieve the worthy goals listed on the next page.
- *Involving patients and families with the care center teams to improve care.* A strong partnership between patients, families and healthcare providers is critical if we are to achieve the best possible outcomes or results. Such a partnership will help tailor care to the needs and preferences of the person with CF. It will also help as people with CF and their families learn to fit CF care into their daily lives.
- *Identifying and enabling "best practices."* The Patient Registry data shows some differences in practice patterns and outcomes across the CF Foundation's Care Center Network. However, the data are not adjusted for factors that we know impact the outcomes, such as family income. The CF Foundation is working with experts to help sort out these complex issues. Adjusting the Patient Registry data will help identify care centers with excellent outcomes. We are planning to have this data available to patients and families by the end of 2006. The next step is to learn how these centers achieve excellent results and share that information with everyone.
- *Providing key information for care teams.* Having information (e.g., current care guidelines, graphs that show change in weight and lung function, etc.) at the time the care team is making decisions helps them to deliver the "best" care. We are working to make this information available to all care centers.

We strongly encourage people with CF and their families to partner with their CF care center team to speed up improvement in CF care. To learn how to get started, watch the Virtual Patient Education Day Web cast "One Team's Story: Raising the Bar for CF Care," and read "It's All About Suzy," available on the CF Foundation's Web site (**www.cff.org**).

Sincerely,

Bruce C. Marshall, M.D.
Vice President of Clinical Affairs
Cystic Fibrosis Foundation

What Is the Cystic Fibrosis Foundation Patient Registry?

Almost 40 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 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 enzyme use, length of hospitalizations, home IVs and complications related to CF.

Information in the Patient Registry helps caregivers and researchers see new trends, design clinical trials to test new therapies and improve the delivery of care for people with CF.

To get the best information, it is important for people with CF to participate in the Patient Registry. The following pages contain information from the Patient Registry relating to the CF Foundation's seven worthy goals to improve CF care. These goals are:

1) To make people with CF and their families full members of the care team;
2) To help people with CF achieve normal growth and nutrition status;
3) To diagnose respiratory infections early and ensure that the right therapies are received;
4) To decrease the spread of germs between people with CF;
5) To prevent complications and/or to diagnose and treat them early;
6) To provide care regardless of race, age, education or insurance coverage; and
7) To support all transplantation and end-of-life care decisions.

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 their normal functions. Many people with CF have a cycle of lung infection or exacerbation and inflammation (swelling). This cycle slowly damages the lungs and their ability 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 3,500 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.

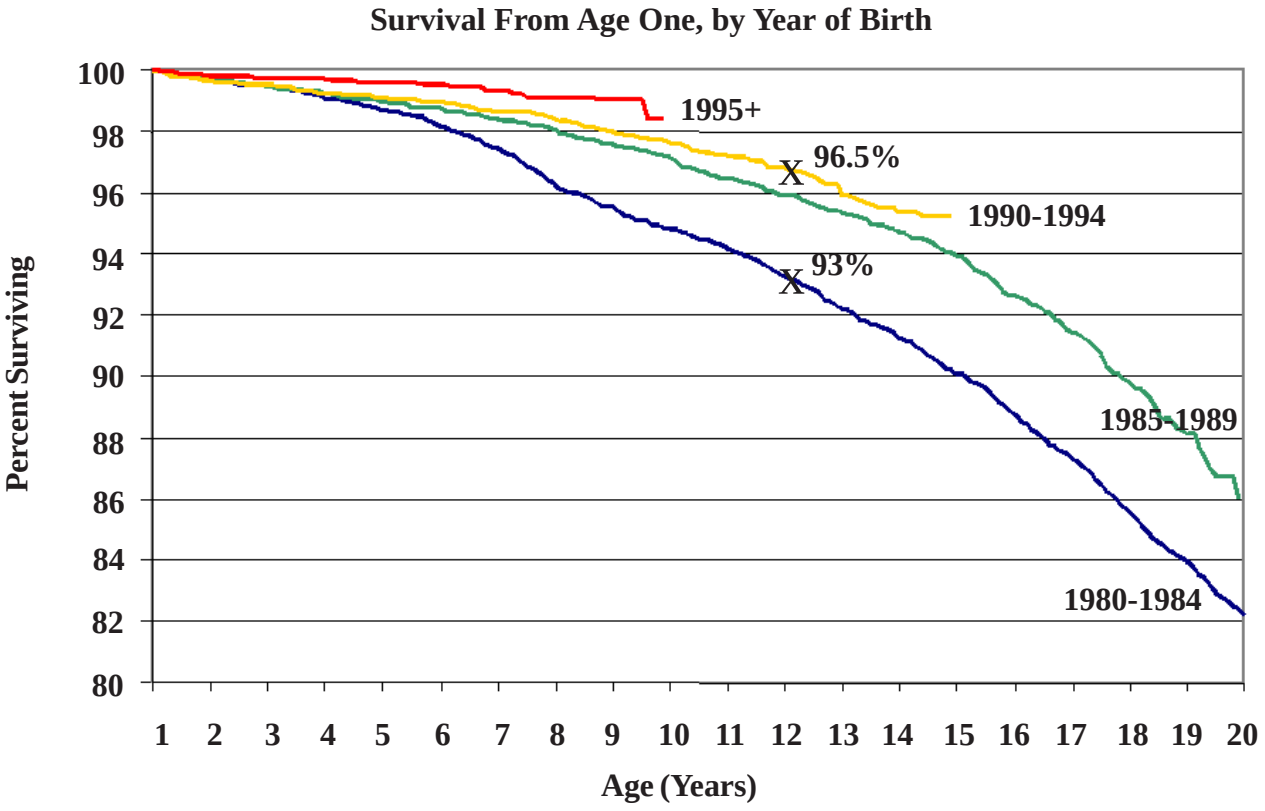
What Is the Cystic Fibrosis Foundation?

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 continue this mission, the CF Foundation has a network of more than 115 accredited care centers across the United States for people with CF. The CF Foundation provides grants, training in quality improvement, and the latest CF care guidelines in the medical literature to support the care 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 and new therapies. To learn more about CF and the CF Foundation, visit **www.cff.org**.

Improving Survival

Because of the hard work of people with CF and their families and CF Foundation-accredited care center teams, the predicted survival for 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 mid 30s. Continued improvement in survival depends, in part, on gathering and using data from people with CF across the United States through the Patient Registry.



The graph above shows that survival of people with CF has improved since 1980. Of people with CF born between 1980 and 1984 (dark blue line), 93 percent were alive at age 12. For children born between 1990 and 1994 (yellow line), 96.5 percent were alive at age 12. Children born in 1995 and after (red line) are doing even better.

Goal 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.

Through the Patient Registry, care centers have reports about their patients. These reports help care centers communicate with their patients and families about some of the important aspects of the disease. They are available from your CF Foundation-accredited care center. The next page is an example of a "Patient Summary Report." It shows trends in lung function, nutrition and other important information about the patient. Additionally, learning about CF from your CF healthcare team and the CF Foundation's Web site (**www.cff.org**) can help you to be more informed when making healthcare decisions.

Patient Report Example:

VISIT DATE: _____ **Name:**
Last Hospitalization: None **Date of Birth:** 12/26/
Last HomeIV Therapy: None **Genotype:** 01/25/1998 ^F508/^F508
Last Clinical Visit: 7/15/2005

Culture Results

Last Culture: Pseudomonas aeruginosa, : 3/29/2005

Last Positive:	**B. cepacia**	**MRSA**	**PA**	**MDR-PA+**
	None	**None**	**3/29/2005**	**10/14/2003**

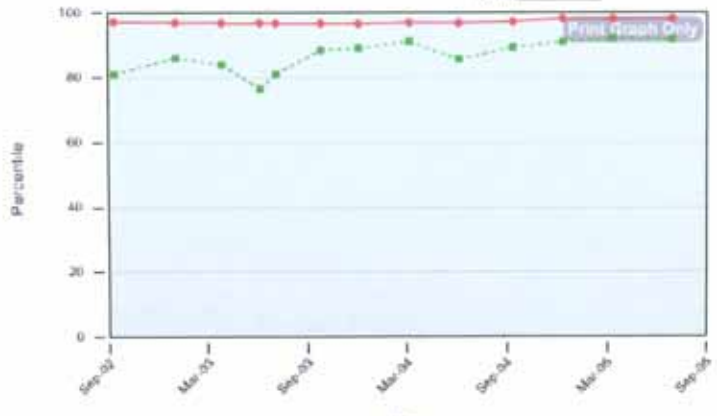
PFT Trend

Date: _____

FEV1
Current Value _____

Nutritional Trend

Date: _____



Height %ile
Current Value _____

Weight %ile
Current Value _____

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

Complications

ACTIVE	RESOLVED
None	• CFRD - w/hyper. (oral agents) • Nasal polyps

Routine Evaluations

Last PFT: 7/15/2005
Last CXR: 12/28/2004
Last SW Visit: None

Last Dietary Visit: 3/29/2005
Last LFT: 12/28/2004
Glucose Screening: 12/28/2004
Creatinine: 12/28/2004

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

Comments

Guidelines for CF Care

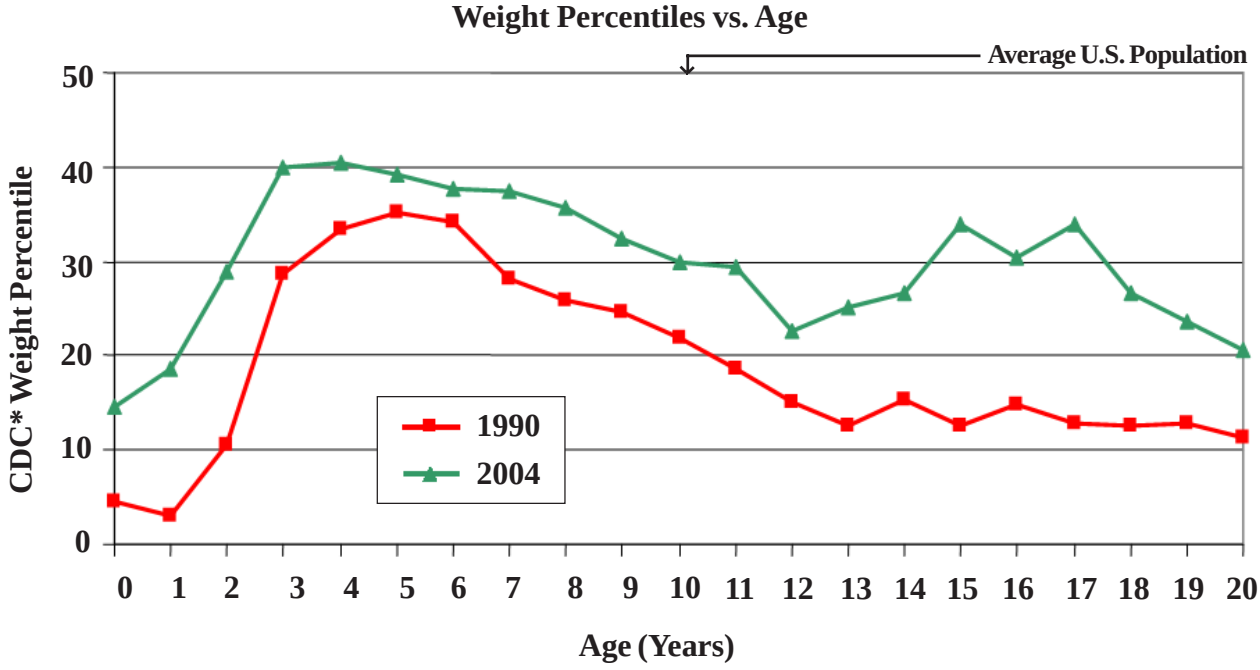
Since the early 1990s, the CF Foundation has gathered experts to review the medical literature on CF care to develop guidelines for the care of all people with CF. Below are the percentages of patients in the Patient Registry who met some of these recommendations in 2004.

Guidelines for CF Care	Children Who Meet Guidelines (%)	Adults Who Meet Guidelines (%)
Clinic Visits — 4 or More Per Year	64.1	51.3
Pulmonary Function Tests — (PFT) 2 or More Per Year	87.0	78.1
Respiratory Cultures — At Least 1 Per Year	94.2	86.8
Creatinine Level — Every Year	76.4	74.7
Glucose — Every Year if ≥14 Years	78.3	73.0
Liver Enzymes — Every Year	76.8	71.9

Maintaining Normal Nutrition

Goal 2: 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 more work remains. The CF Foundation continues to work with CF care experts to update the guidelines for treating and managing the nutrition of everyone with CF. The Patient Registry shows a strong link between good nutrition and survival.

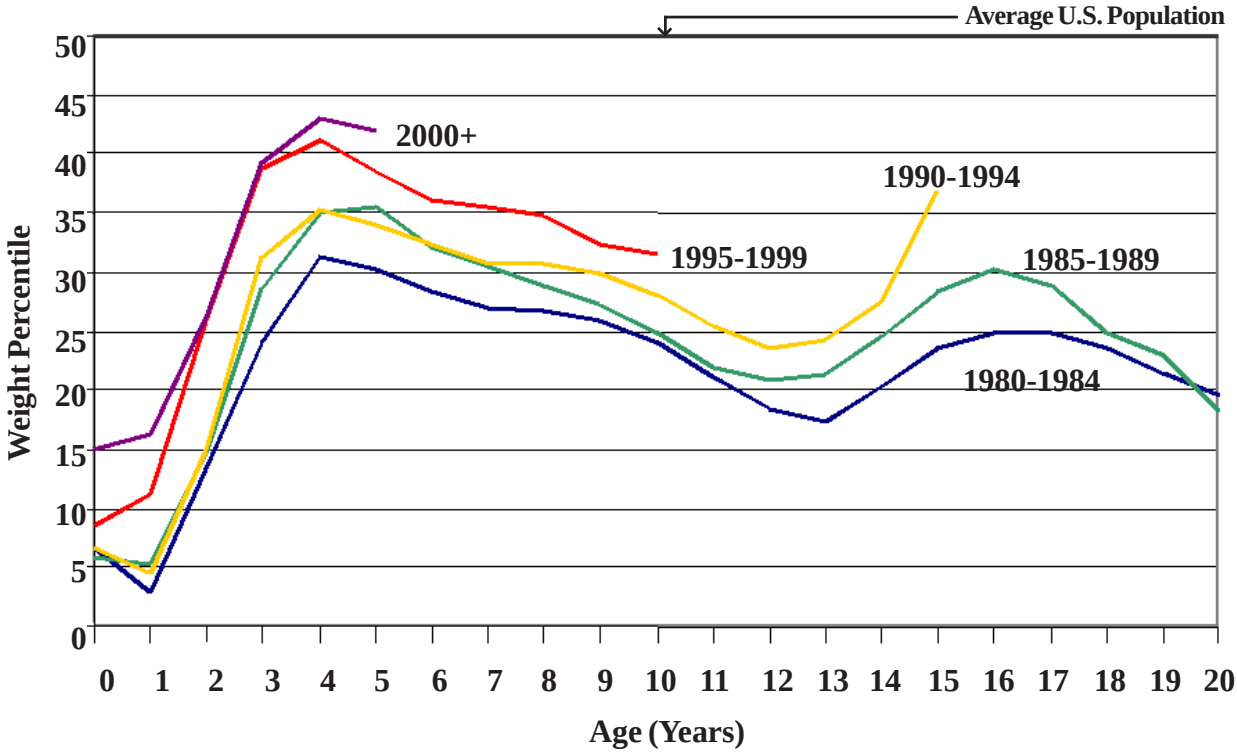


* Centers for Disease Control and Prevention — www.cdc.gov/growthcharts.

The previous graph shows how much the weight of children with CF has improved since 1990. However, the goal is for them to reach at least the 50th percentile, the average weight of children in the United States. Children with CF who have good nutrition and weight will most likely grow to a height close to their parents' height. CF care center teams are working hard to help all children with CF grow normally.

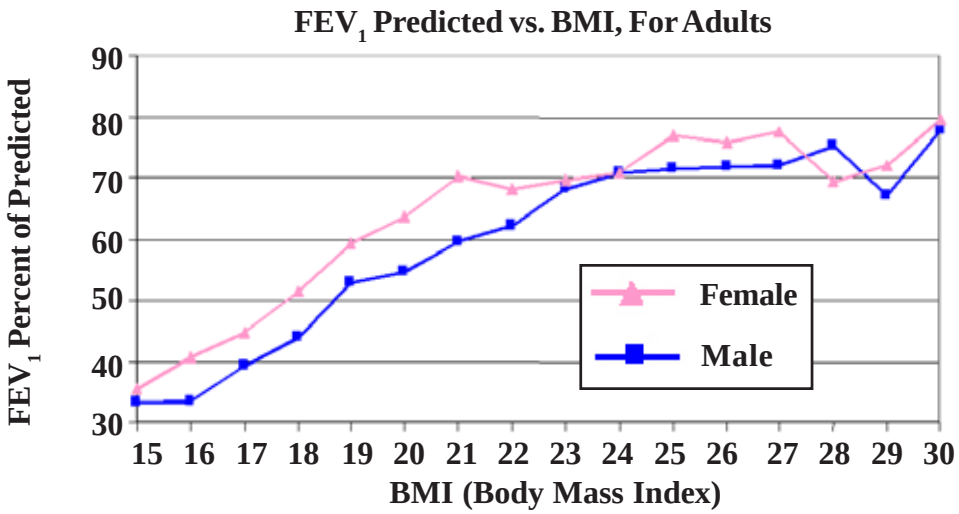
Good nutrition can help keep lungs healthy. The following graph shows steady improvement in the nutrition of children with CF. Of people with CF born between 1980 and 1984 (dark blue line), the highest average weight was at the 30th percentile at age 4 compared to U.S. population of the same age. For children born between 1995 and 1999 (red line), the highest average weight was above the 40th percentile at age 4 compared to the U.S. population of the same age.

Median Weight Percentile vs. Age by Year of Birth



Keeping the best possible nutritional status is also important for adults with CF. In adults, body mass index (BMI) is used as a measure of nutrition health. BMI is calculated by dividing body weight in kilograms by the person's height in meters squared (weight kg/height m^2 = BMI). For adults with CF, BMI and lung function appear to be related. Lung function is measured by FEV_1, or Forced Expiratory Volume over one second, which is shown as percent predicted based on healthy, non-smoking people. As shown in the first graph on the next page, a higher BMI is related with better FEV_1. You can calculate your BMI on the Centers for Disease Control and Prevention's Web site (www.cdc.gov/nccdphp/dnpa/bmi/index.htm).

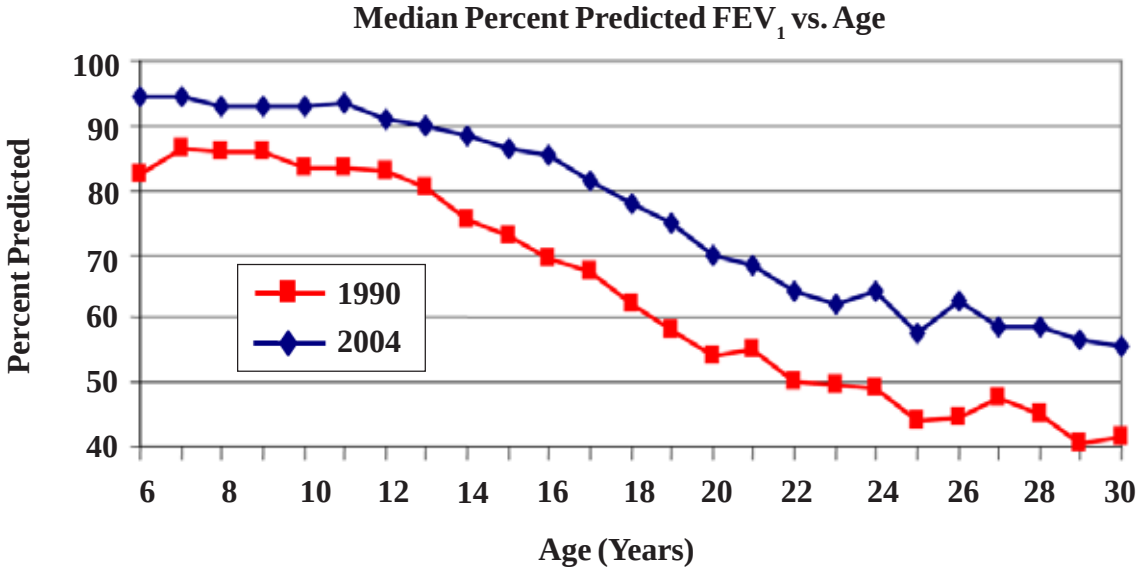
To learn more about CF and nutrition, ask your CF care team or read about nutrition and CF on the CF Foundation's Web site at **www.cff.org**.



Lung Function

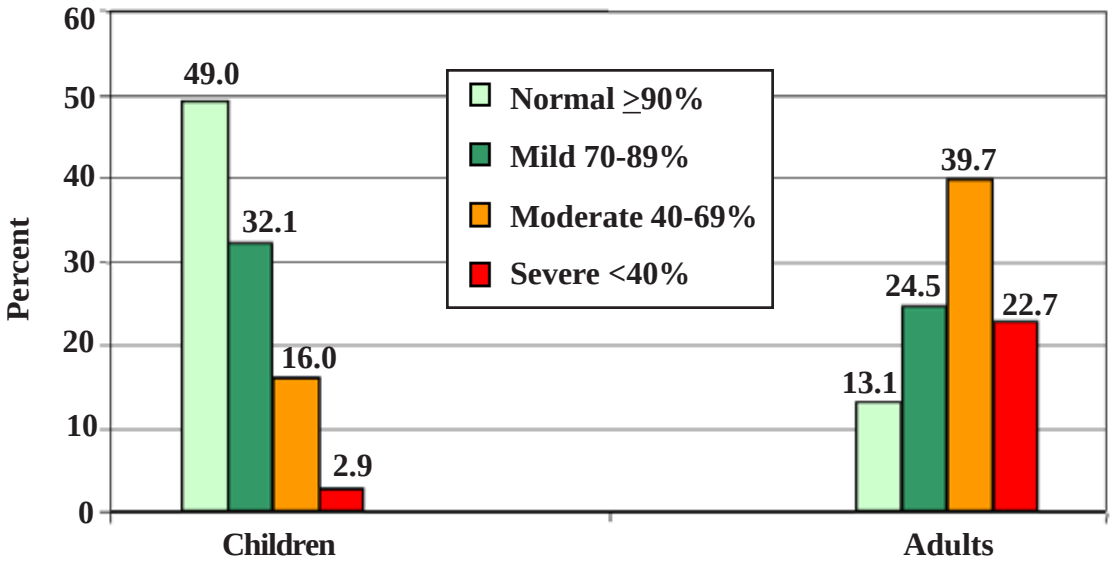
Goal 3: Everyone with CF will receive the right therapies to keep lung function steady and to decrease the number of pulmonary exacerbations or respiratory infections. Exacerbations will be diagnosed early and treated appropriately.

Everyone with CF starts with healthy lungs at birth. FEV_1 (lung function) is usually near normal or just under 100 percent predicted when first measured around 6 years of age. The graph below shows major improvement in lung function since 1990.



The next graph shows the percentage of children and adults and the severity of lung disease in each group. The lower a person's FEV_1, the more severe the lung disease. Doing airway clearance and exercise helps keep lungs healthy. These along with better nutrition, antibiotics like TOBI® and azithromycin and therapies like Pulmozyme® all work to improve FEV_1 and lung health in people with CF. To learn more about what you or your child can do to keep the lungs healthy, watch the Web cast "Total Lung Care: Feel the Difference T.L.C. Makes," on the CF Foundation's Web site (**www.cff.org**).

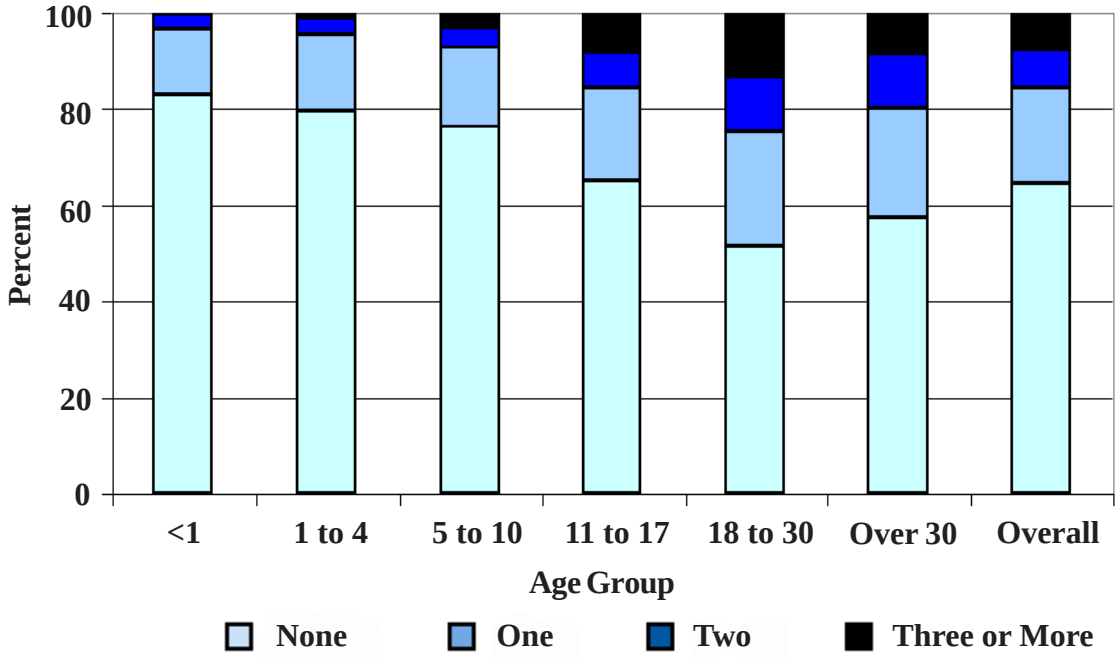
Severity of Lung Disease for Children and Adults — FEV_1 Percent Predicted



Each time a person has a respiratory infection, there may be lung damage. However, there are things you can do to decrease the chance of lung damage, such as: (1) Getting a flu shot every fall for you/your child and everyone living in the house; (2) Doing airway clearance to keep the lungs as clear as possible of mucus; (3) Taking the medicines as prescribed by your CF care team; (4) Telling your CF care team early when you or your child do not feel well; and (5) Avoiding germs by using good hand hygiene and cleaning and disinfecting equipment.

Diagnosing and treating pulmonary exacerbations or respiratory infections quickly also is important. The graph below shows the percentage of people with CF who had exacerbations that were treated in the hospital or at home with intravenous (IV) antibiotics in 2004. In general, more exacerbations occur in adolescents and adults.

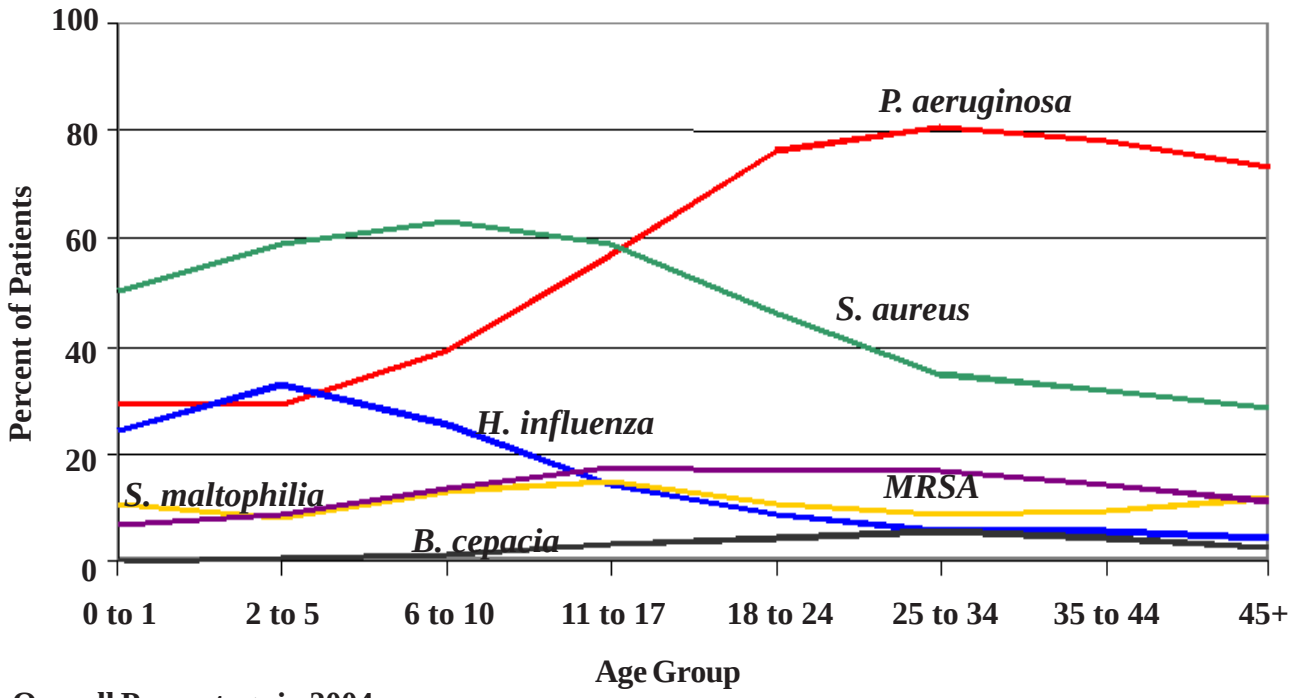
Pulmonary Exacerbations in 2004 vs. Age



Goal 4: People with CF and their care teams will work together to eliminate the chances of patients getting respiratory pathogens or germs, particularly *Pseudomonas aeruginosa* and *Burkholderia cepacia* complex, in the hospital, clinic and home.

Repeated pulmonary exacerbations are a concern for people with CF. It is the cycle of infection and inflammation (swelling) that may damage the lungs. This damage causes lung function (FEV_1) to decline. When the lungs are damaged, exacerbations happen more often. The next graph shows the germs that are usually found in the lungs of people with CF. Talk to your CF care team to learn more about preventing respiratory infections. Information about what CF germs are, how they are spread and how to avoid germs is available on the CF Foundation's Web site (**www.cff.org**) and discussed in the Web cast "How to Avoid Germs in CF."

Respiratory Infections vs. Age



Overall Percentage in 2004:

- *P. aeruginosa* 57.3%
- *S. aureus* 51.7%
- *H. influenza* 16.2%
- *S. maltophilia* 11.6%
- *B. cepacia* complex 2.9%
- *MRSA* 14.6%

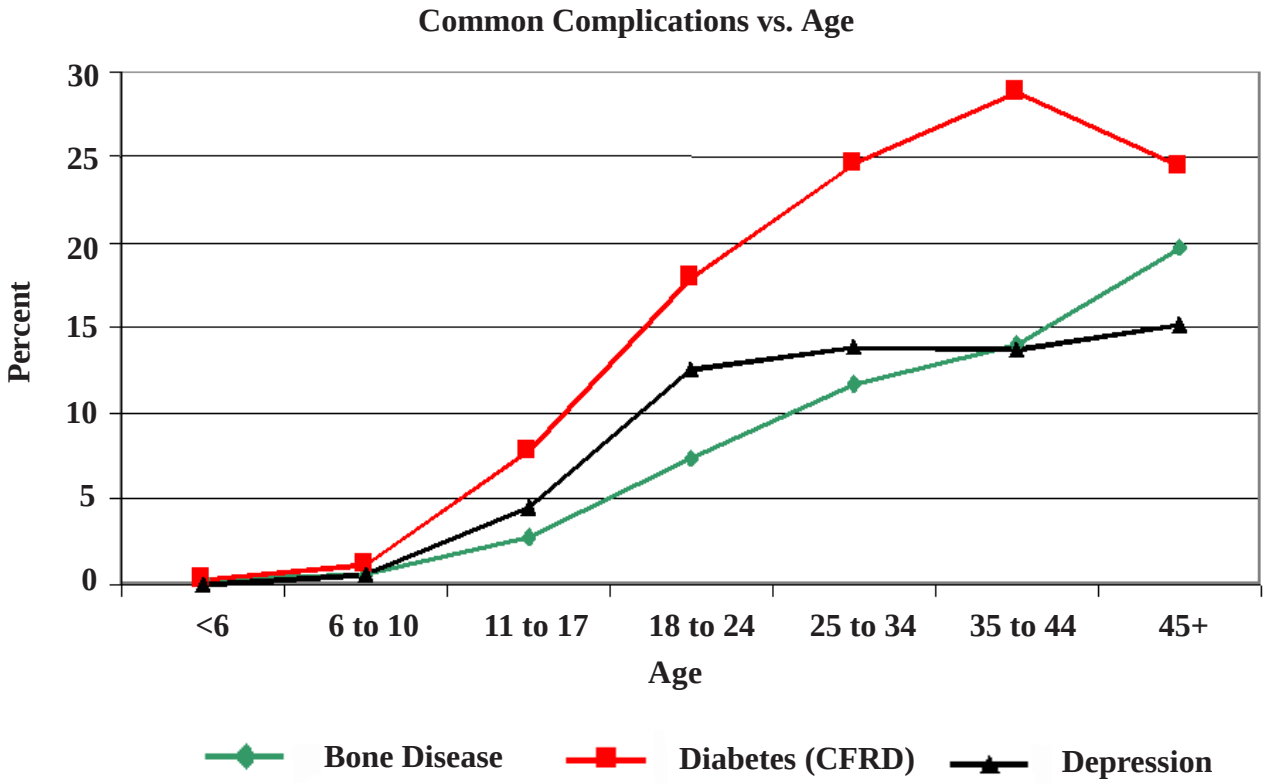
Complications of CF

Goal 5: People with CF will be closely monitored for complications of CF, especially CF-related diabetes (CFRD). For any complication, prevention and early treatment when possible should be the approach.

In the early 1990s, data in the Patient Registry showed an increase in the number of teens and adults with diabetes. CFRD was found to be different from diabetes in people without CF. The CF Foundation brought together experts in CF and diabetes and developed guidelines for this problem. The guidelines outline how to test for and treat CFRD. Anyone with CF, 14 years of age or older, should be tested every year for CFRD. Data suggests that diagnosing and treating CFRD earlier results in better outcomes. The CF Foundation continues to fund CFRD research. To learn about CF Foundation-supported research, visit **www.cff.org** and click on "Research and Clinical Trials."

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 show that about 10.7 percent of people with CF had bone disease in 2004. Preventing or lessening bone disease begins in childhood when bones are growing. Ask your CF dietitian what can be done to keep your or your child's bones healthy.

Another finding from the Patient Registry is that more than 13 percent of adults with CF have symptoms of depression. This is a common complication of many chronic diseases. People with CF, their families and caregivers need to be aware of this complication, so that diagnosis and treatment can be started early. People often respond well to treatment for depression. The CF Foundation continues to promote prevention, early diagnosis and treatment of complications that people with CF may experience.



Access to Care

Goal 6: Everyone with CF will be able to receive appropriate therapies, treatments and support regardless of race, age, education or insurance coverage.

Research data suggests that people with CF who live in households with lower incomes are more likely to have poorer lung function and worse nutrition. 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 show that people with CF, no matter their income, have therapies like Pulmozyme® and TOBI® available to them. One way this is done is through CF Services, a mail-order pharmacy and wholly owned subsidiary of the CF Foundation (**www.cfservicespharmacy.com**).

Learn more about race and age of the people in the Patient Registry by turning to page 12, "A Summary of the 2004 Data."

Insurance*	Children (%)	Adults (%)
No Insurance	0.8	2.1
Private/HMO	62.2	65.2
Medicaid/State	46.3	33.3
Federal/CHAMPUS	2.9	13.1

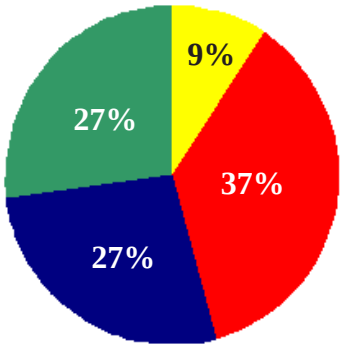
*Data are not mutually exclusive, as people with CF may have more than one type of insurance.

Adults With CF

In 1990, about 30 percent of people in the Patient Registry were age 18 or older. In 2004, more than 41 percent of people with CF in the Patient Registry were adults, and the number continues to grow. Because of this trend, the CF Foundation brought together experts in CF and adult medicine and developed guidelines for the care of adults with CF. The CF Foundation also mandated the development of adult care programs. It is important to help teens transition from depending on their parents or another adult to taking charge of their own healthcare.

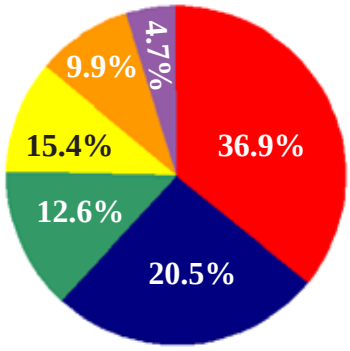
Data from the Patient Registry show some facts about adults with CF. As you can see, they lead busy and productive lives while dealing with CF.

Education



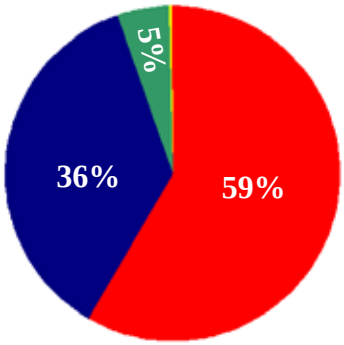
- Less Than High School
- High School Diploma
- Some College
- College Graduate

Employment



- Full-time
- Part-time
- Student
- Homemaker
- Unemployed
- Disabled

Marital Status



- Single
- Married/Living Together
- Separated/ Divorced
- Widowed (0.2%)

CF and Pregnancy

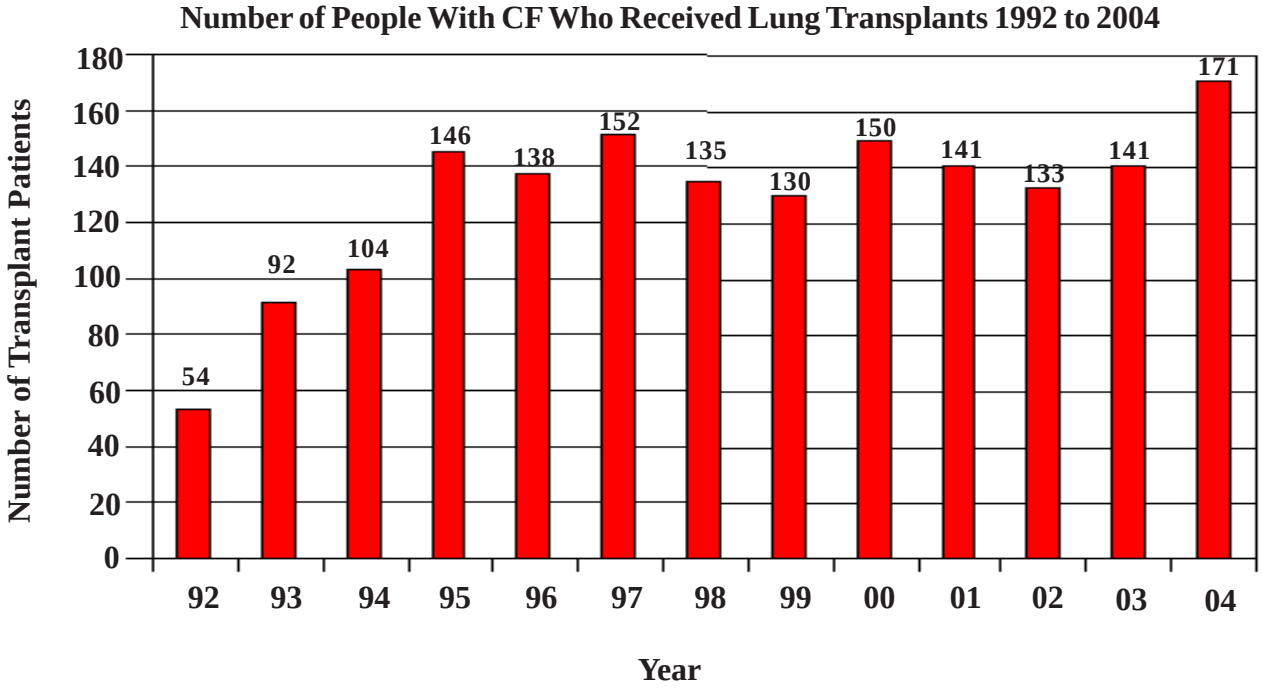
Many adults with CF wish to have children. In the 1980s, it was thought to be too risky for a woman with CF to get pregnant. Now, thanks to improvements in survival and research done with Patient Registry data, CF care teams can give better advice about the risks of pregnancy. In 2004, the Patient Registry reported that 191 women with CF were pregnant.

Also, with advances in fertility medicine, more men with CF are able to father children than ever before.

Transplantation and End-of-Life Care

Goal 7: Everyone with CF will be supported by their CF team when making decisions about transplantation and end-of-life care.

Having severe lung disease often leads people with CF to think about a lung transplant. Unfortunately, a lung transplant is very risky and the supply of good lungs to transplant is limited. It is important to understand who can benefit from this procedure and when is the best time. The Patient Registry data have been used to help identify who is most likely to benefit from a transplant. Almost 1,800 people with CF have received lung transplants since 1988. The guidelines for deciding who gets donor lungs changed in 2005. The CF Foundation will be monitoring how these changes will affect people with CF. To learn more about lung transplants and organ donation, visit the CF Foundation's Web site at **www.cff.org**.



Who Are the CF Patients in the Patient Registry — A Summary of the 2004 Data.

CF patients (number)	22,714
Newly diagnosed patients in 2004 (number)	909
Age at diagnosis (median)	6 months
Age range	0 to 74 years
Total number of deaths	354
Predicted median survival	35.1 years
Patients 18 years and older (%)	41.8
Race/Ethnicity (%)	
Caucasian	95.3
Hispanic (black or white)	6.7
African American	4.0
Males (%)	52.1
Genotyped (%)	83.8
Home therapy (%)	
IV antibiotics	20.1
Oxygen	6.3
Supplemental feeding – tube	9.1
oral only	36.1
Taking pancreatic enzyme supplements (%)	89.9
Clinical trial participation (number)	1,694
FEV_1 % predicted (mean)	75.1
Patients with weight <5th percentile* (%)	15.7
Patients with height <5th percentile* (%)	16.3
Respiratory cultures positive for (%)	
P. aeruginosa	57.3
B. cepacia complex	2.9
S. aureus	51.7
S. maltophilia	11.6
MRSA	14.6
Complications (%)	
Diabetes (CFRD)/glucose intolerance	16.1
Bone disease (patients ≥ 18 years)	10.3
Liver disease	7.9
Nasal polyps	4.1
Transplants (numbers)	
Lung:	
Bilateral	160
Lobar-cadaveric	7
Lobar-living related donor	10
Liver:	18
Therapies**	
TOBI®	67.5
Pulmozyme®	72.4
Ibuprofen	4.7
Macrolides (e.g., azithromycin)	49.3
Total pregnancies among women aged 13 to 45 (number)	191
Live births (per 100 women age 13 to 45)	1.8

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

**This is the percentage of patients who are eligible for a therapy and had it prescribed at least once in 2004.

Number of Patients by State in the CF Patient Registry

State	Number	Percent
Alabama	335	1.5
Alaska	57	0.3
Arizona	335	1.5
Arkansas	218	1.0
California	1,856	8.2
Colorado	432	1.9
Connecticut	274	1.2
Delaware	45	0.2
District of Columbia	21	0.1
Florida	1,081	4.8
Georgia	652	2.9
Hawaii	11	<0.1
Idaho	139	0.6
Illinois	909	4.0
Indiana	538	2.4
Iowa	272	1.2
Kansas	244	1.1
Kentucky	430	1.9
Louisiana	277	1.2
Maine	177	0.8
Maryland	442	1.9
Massachusetts	801	3.5
Michigan	861	3.8
Minnesota	476	2.1
Mississippi	209	0.9
Missouri	558	2.5
Montana	100	0.4
Nebraska	200	0.9
Nevada	127	0.6
New Hampshire	177	0.8
New Jersey	509	2.2
New Mexico	87	0.4
New York	1,472	6.5
North Carolina	660	2.9
North Dakota	52	0.2
Ohio	1,274	5.6
Oklahoma	241	1.1
Oregon	278	1.2
Pennsylvania	1,198	5.3
Puerto Rico	39	0.2
Rhode Island	94	0.4
South Carolina	295	1.3
South Dakota	88	0.4
Tennessee	445	2.0
Texas	1,382	6.1
Utah	264	1.2
Vermont	112	0.5
Virgin Islands	1	<0.1
Virginia	667	2.9
Washington	482	2.1
West Virginia	163	0.7
Wisconsin	582	2.6
Wyoming	51	0.2
Foreign	24	0.1