Cystic Fibrosis Registry of Turkey Data for 2023 - Summary Report

Preface

In this report, we present a summary of the characteristics of our cystic fibrosis (CF) patients followed up in Turkey in 2023.

The "Cystic Fibrosis Registry of Turkey" (CFRT) was established by the "Pediatric Respiratory Diseases and Cystic Fibrosis Association" which annually records disease-related information of CF patients followed up in various centers in our country. This information helps us to better understand CF disease, to have more information about our patients' disease-related characteristics, quality of life and natural course of the disease, to compare it with our previous knowledge, to see the changing numbers over time and to identify patients who will benefit from special or preventive treatment methods in our country.

You can find more detailed information about the CFRT on our website
https://www.kistikfibrozisturkiye.org/hasta-kayit-sistemi/

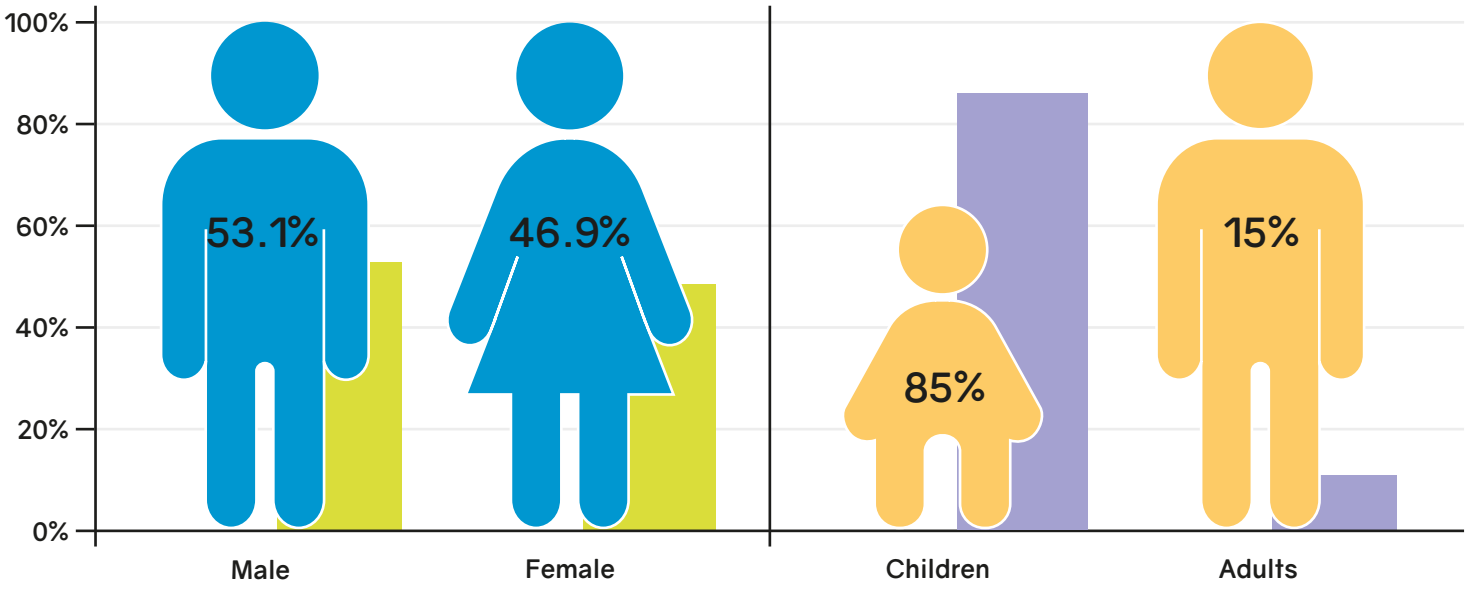
Our detailed report for 2023 is available at
https://www.kistikfibrozisturkiye.org/wp-content/uploads/2024/11/2023-UKKS-2.pdf.

1. Number and Distribution of Patients

2258 CF

In our country, there were 2258 CF patients from 34 centers registered in the CFRT in 2023.

Gender

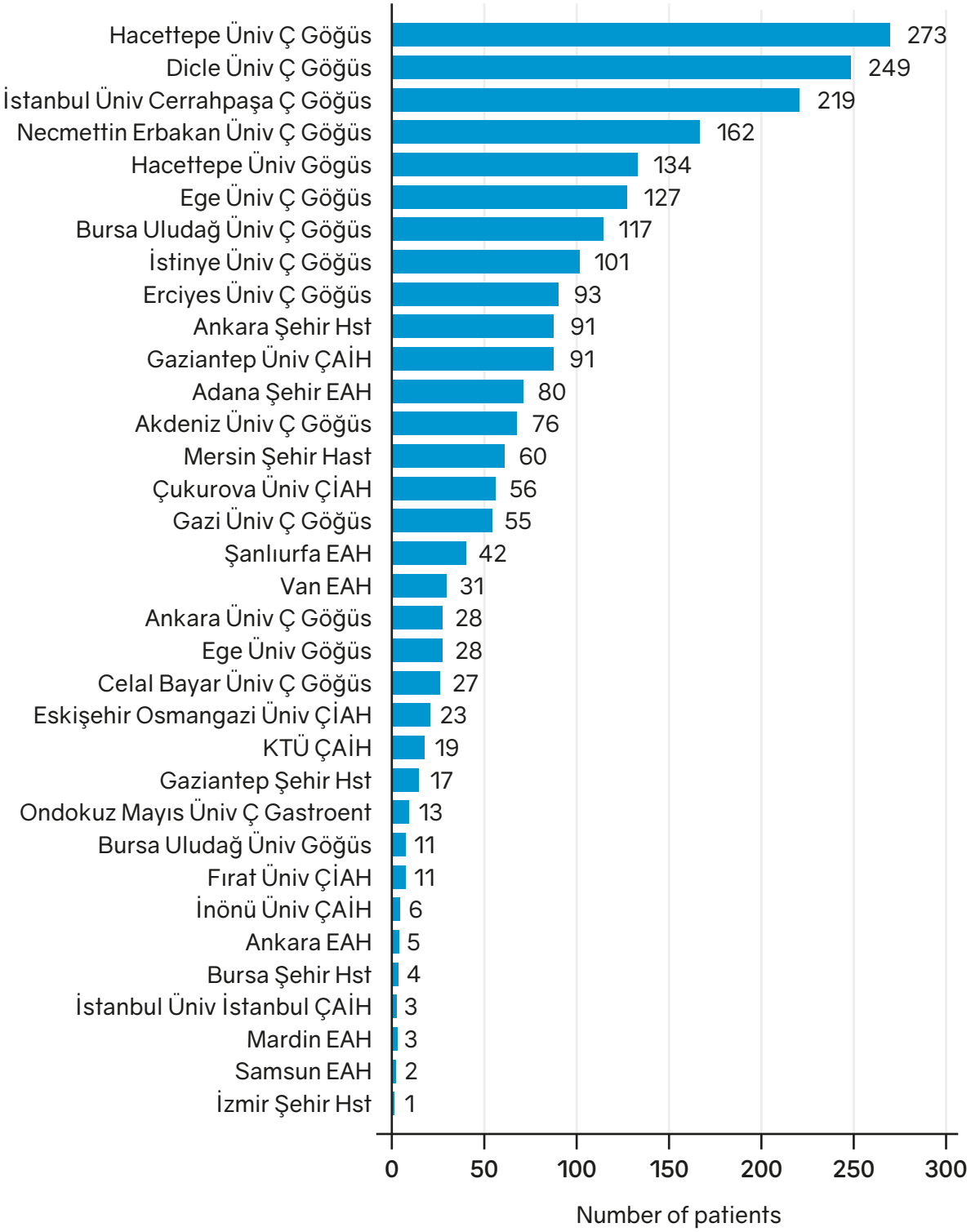


There were 53.1% male and 46.9% female patients.

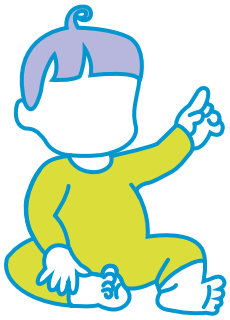
Distribution of patients

85% were children and 15% were adults.

Number of patients registered by centers



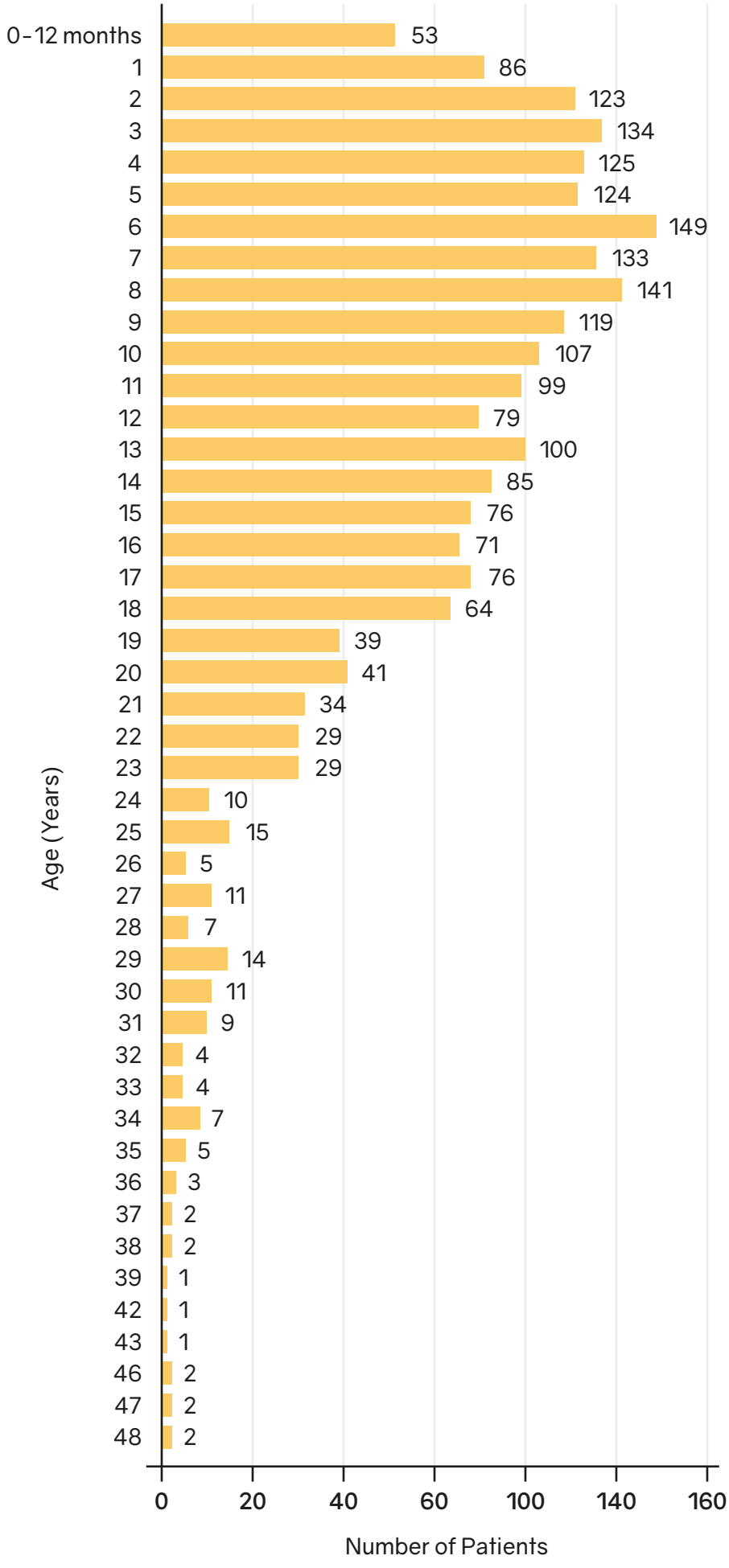
Ç: Pediatric pulmonology
ÇİAH: Pediatric Immunology and Allergy
EAH: Training and Research Hospital



Median diagnostic age for CF patients is

3.9 months

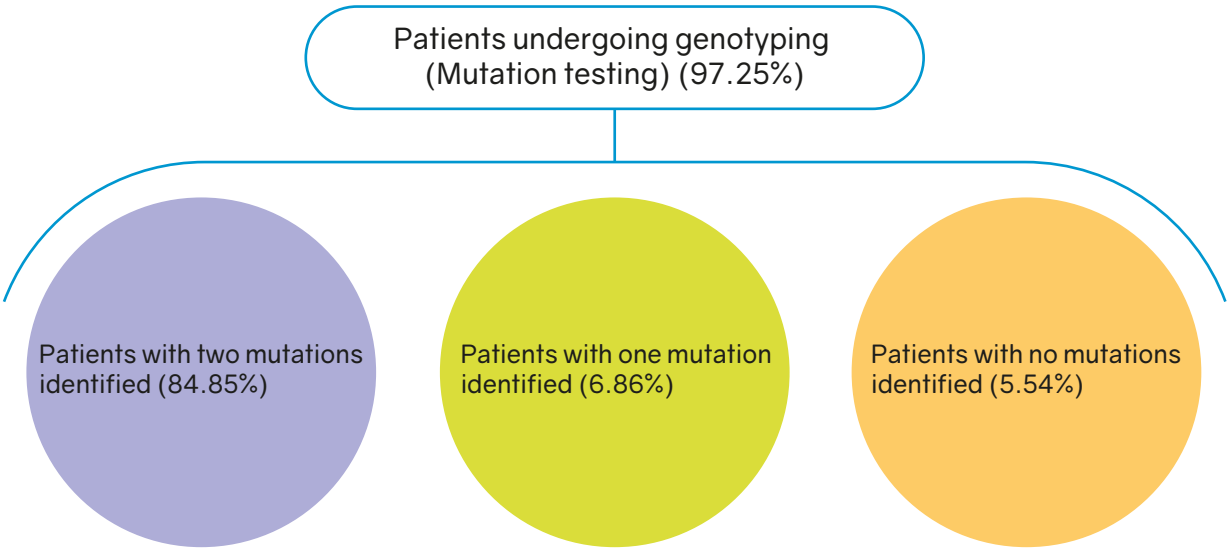
Age distribution of patients



2. Genetics

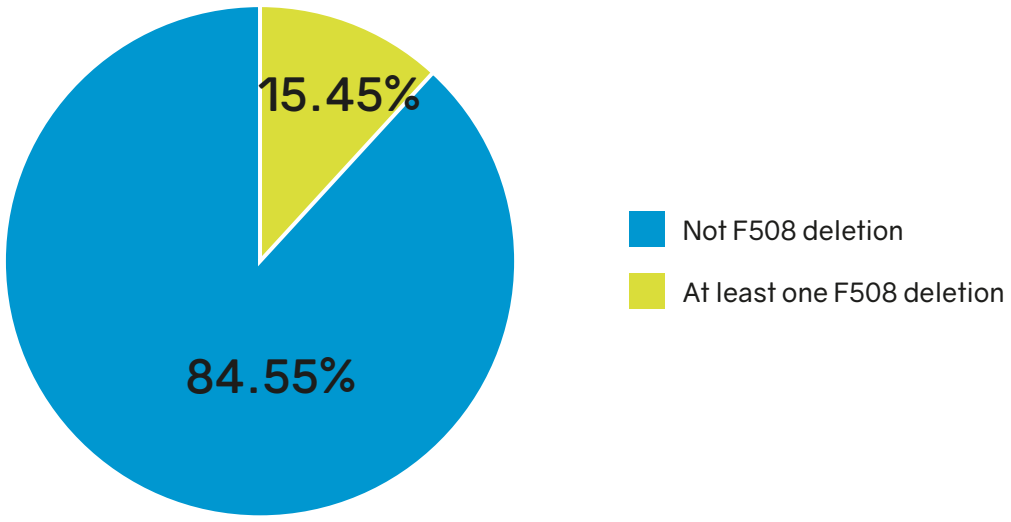
- CF is a genetic disease. Individuals with CF have two CF-causing mutations, one inherited from the mother and the other from the father.
- Genetic test is necessary for definitive diagnosis.

Genotyping results of patients

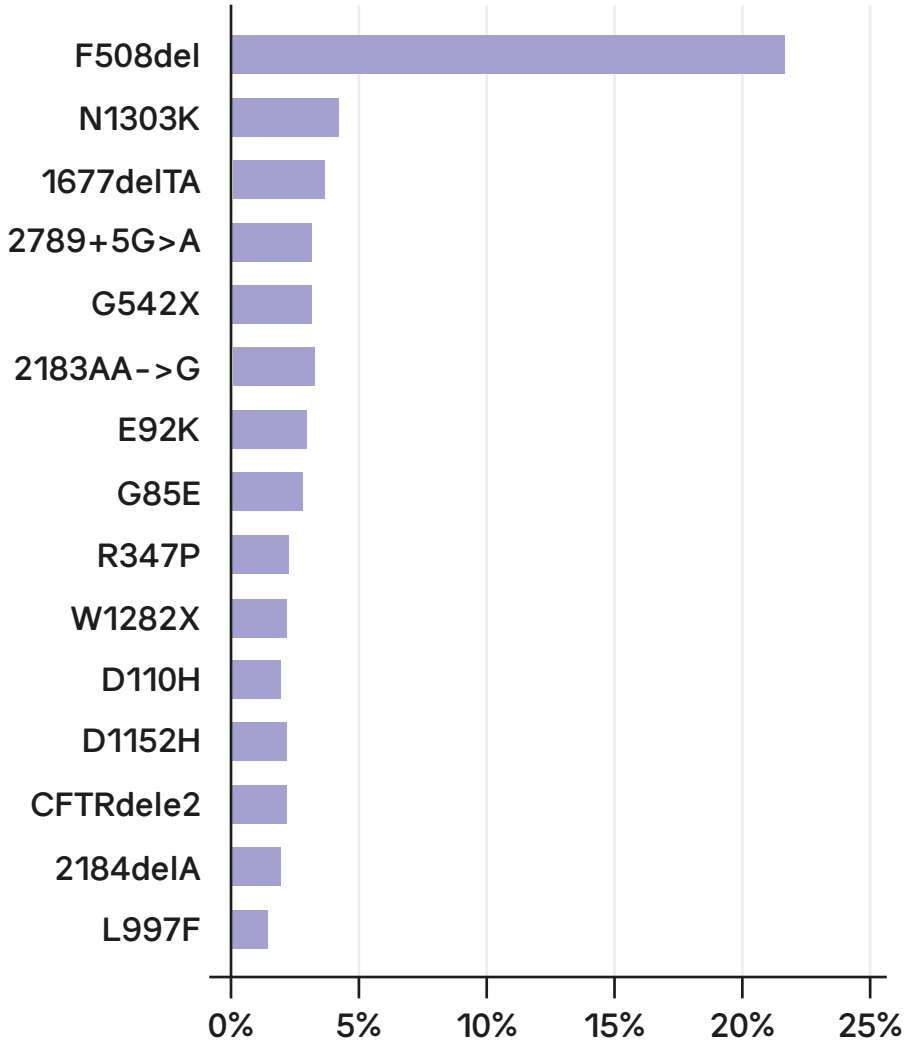


- F508 deletion (F508del) is the most common CF-causing mutation in Europe.
- In Europe, 80% of patients has at least one F508del mutation
- In our country, only 15.45% of patients have at least one F508del mutation

Mutations



Most common mutations in genotyping



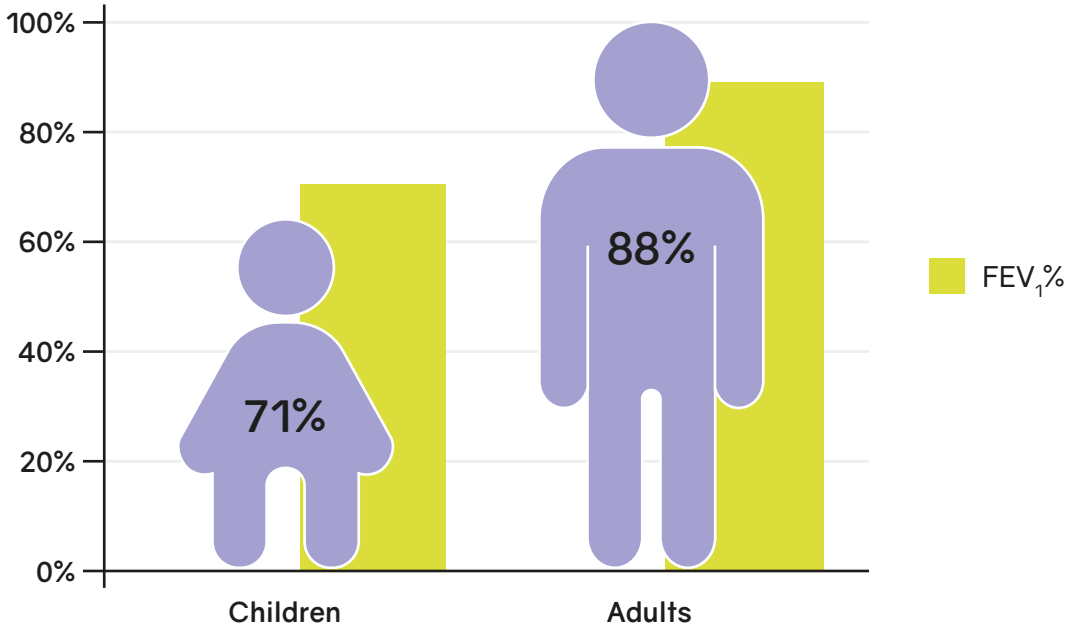
3. Pulmonary Functions

- Pulmonary involvement in CF is the most important factor determining the clinical severity of the disease and the duration and quality of life, therefore, close monitoring of pulmonary functions is important.
- Pulmonary function tests can be performed in patients over 6 years of age and are evaluated by measuring FEV_1.

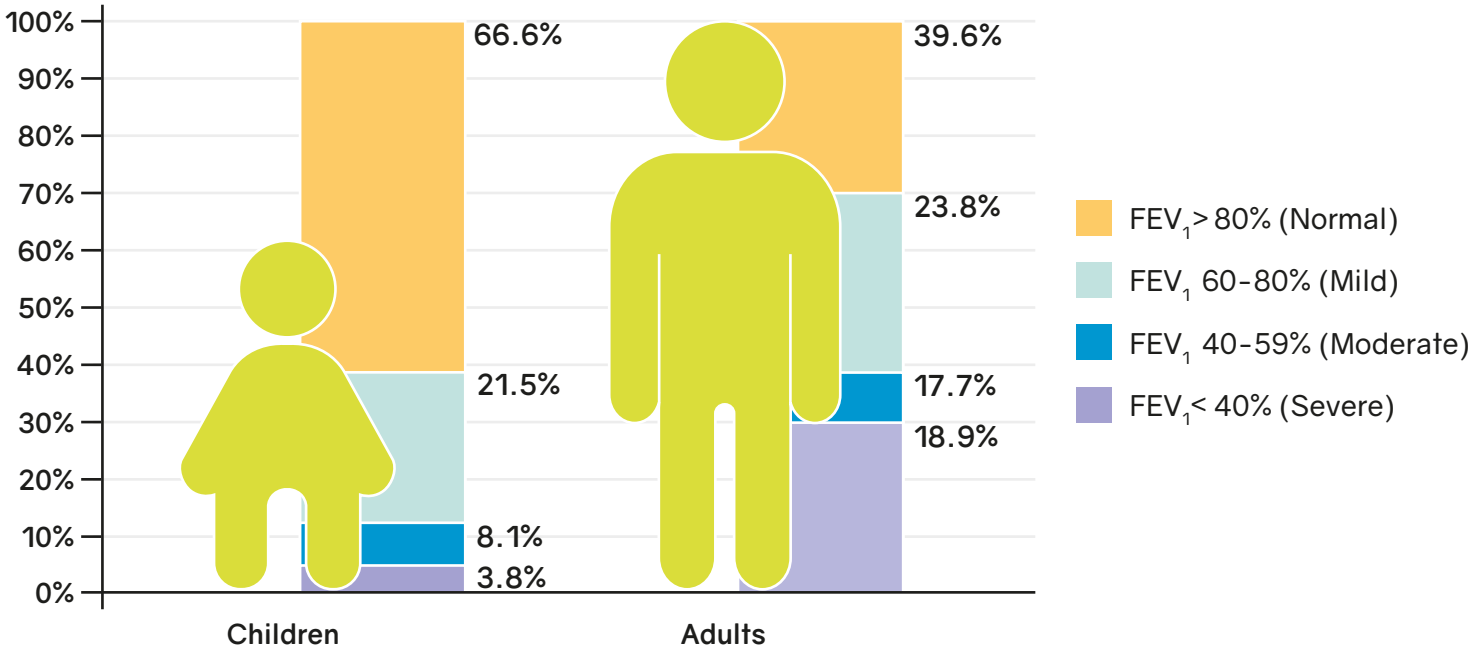
The severity of lung disease is determined according to FEV_1 percentage values:

- Normal: 80% and above
- Mild disease: 60-79%
- Moderate disease: 40-59%
- Severe disease: 39% and below

Median FEV_1% percentages of children and adults



Distribution of children and adults according to FEV_1% values



4. Nutrition

- Digestive problems occur in 85% of CF patients as a result of the inability of pancreatic secretions (enzymes) to be released due to the disease or to flow into the intestines due to obstructions in the ducts.

- Since CF patients cannot digest carbohydrates, proteins and fats, they have abundant, loose-fatty, light-colored, foul-smelling and numerous stools, abdominal distension and gas, and if left untreated, individuals cannot gain enough weight and there is retardation in growth and development.

- Good nutrition in CF is important to maintain pulmonary functions.

- Body mass index, height and body weight z scores are used to monitor nutrition. Low values of these values indicate that the nutritional status of the individual is inadequate.

- Z score is a score that indicates how much a value differs from healthy people of similar age and gender. Since standard values vary for children in different age groups, z scores are used to assess the growth. When this score is below zero, it means that it is low compared to people of similar age and gender.

Body Mass Index (BMI):

- BMI is calculated by dividing body weight by the square of height.

BMI = Body weight (kg) / Square of height (m2)

- BMI assessment:

  2-18 years of age: z-scores are more commonly used.

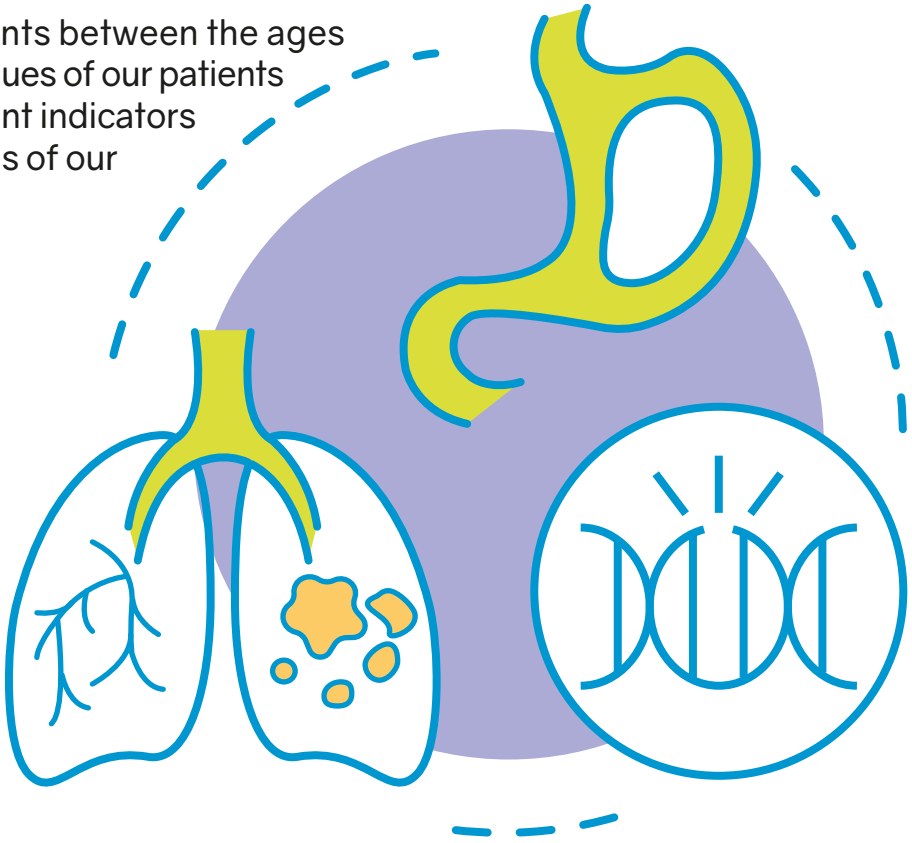
  Over 18 years of age: The formula-calculated value of BMI is used:

  Low: 18.5 and below

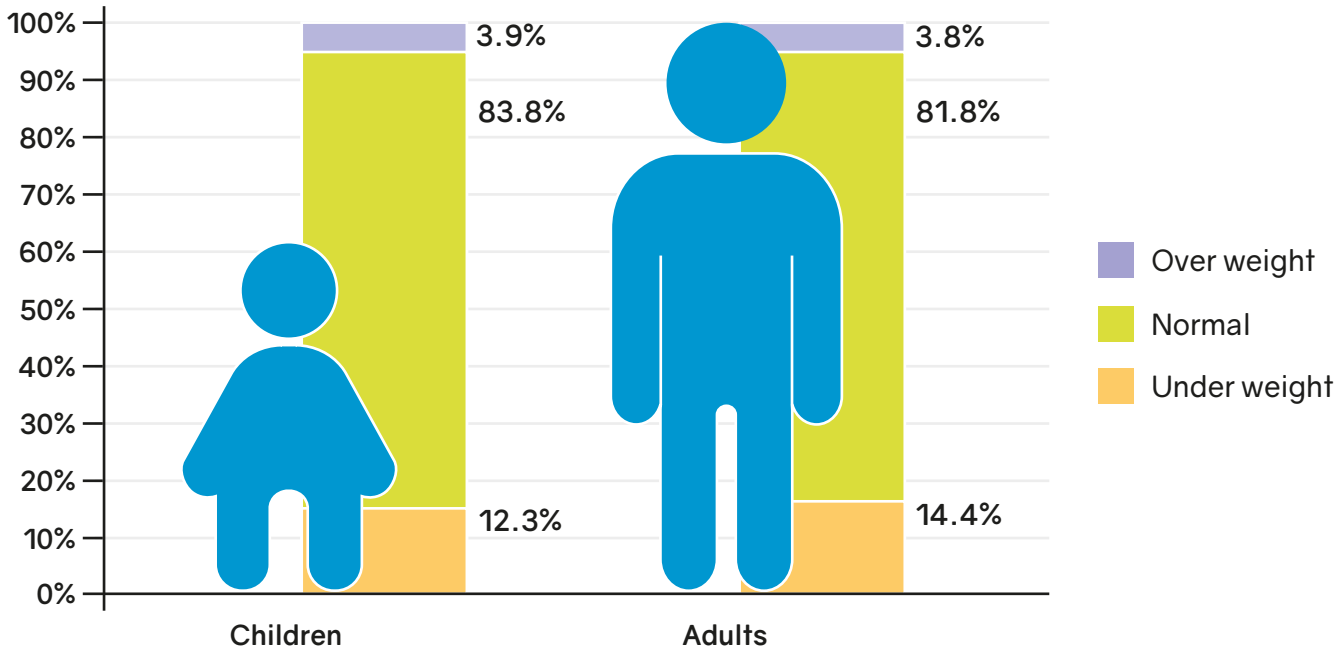
  Normal: 18.5-24.9

  High: 25 and above

- The low BMI z score of our patients between the ages of 2 and 18 years and the low BMI values of our patients aged 18 years and older are important indicators that the growth and nutritional status of our patients are not adequate.



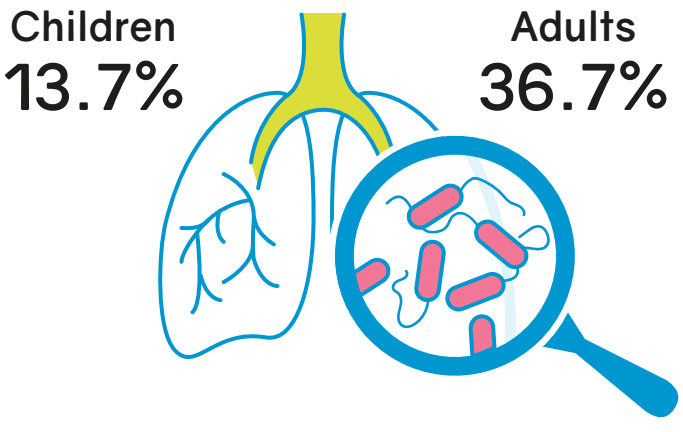
Distribution of BMI and BMI z scores in children and adults



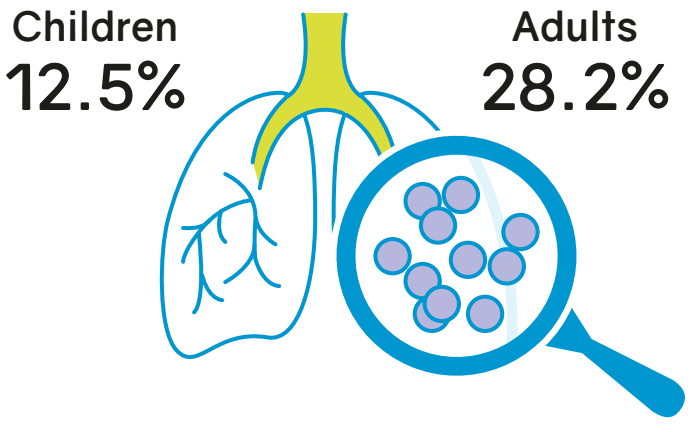
5. Microbiology

- Mucoid secretions in the lungs of CF patients cause airway obstruction resulting impaired lung ventilation. Some microorganisms easily settle in this environment and frequent lung infections occur from an early age.
- The most common microorganisms that settle in the lungs of individuals with CF are *Staphylococcus aureus* and *Haemophilus influenzae* at younger ages and *Pseudomonas aeruginosa* at older ages. *Pseudomonas* in particular causes chronic (long-term) infection of the lungs and is the most important cause of ongoing lung damage.
- Infection control is an important issue as infection transmission is known among individuals with CF.

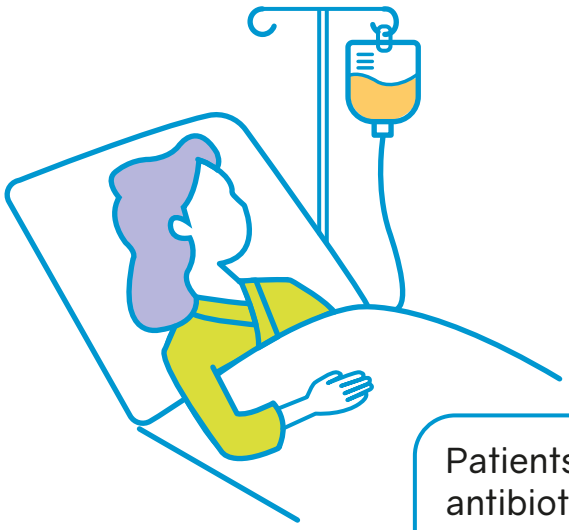
Chronic *Pseudomonas aeruginosa*



Chronic Methicillin sensitive *Staphylococcus aureus*



Hospitalization:



Patients hospitalized (due to CF) to receive intravenous antibiotics for at least 1 day:

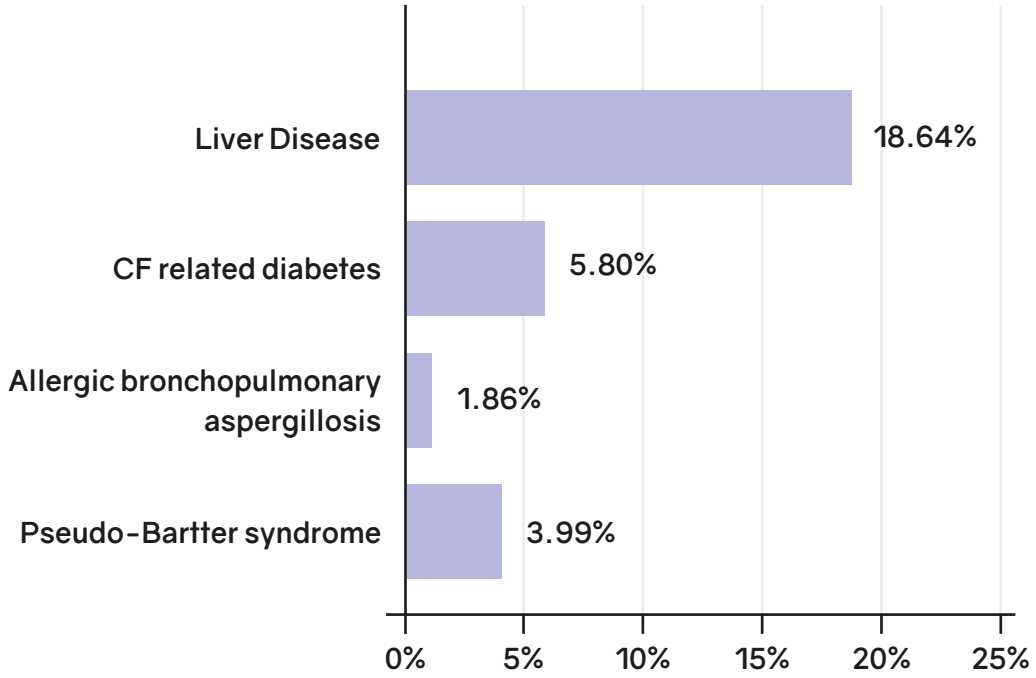
18.38%

6. Complications

- The lungs, pancreas, intestines, liver, sinuses and reproductive organs are affected to varying degrees in CF.

- Complications of CF can include liver disease, diabetes, bone mineralization disorders (osteoporosis) and Pseudo-Bartter syndrome (salt loss).

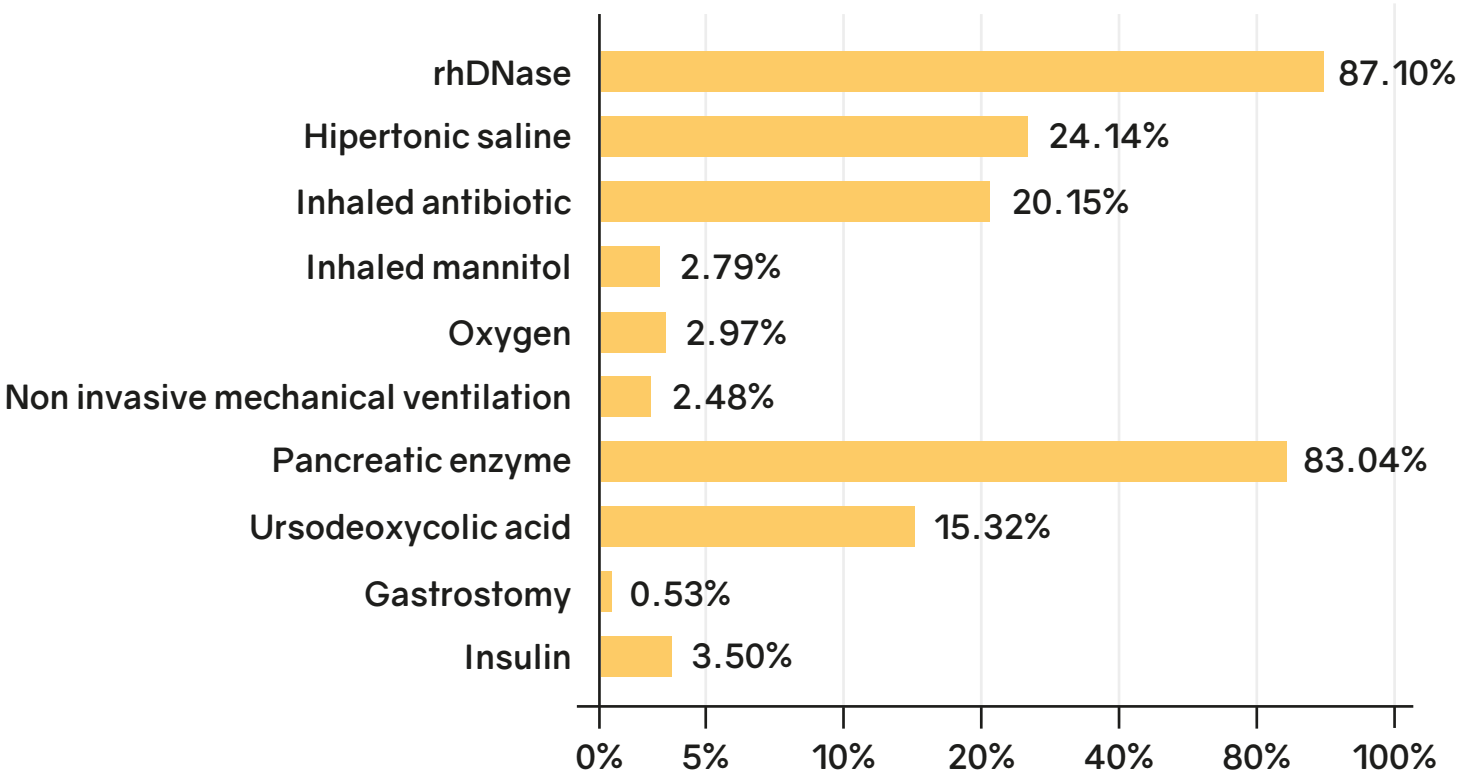
Complications



7. Therapies

- Therapies in CF aim to decrease or improve symptoms and prevent complications related with disease.

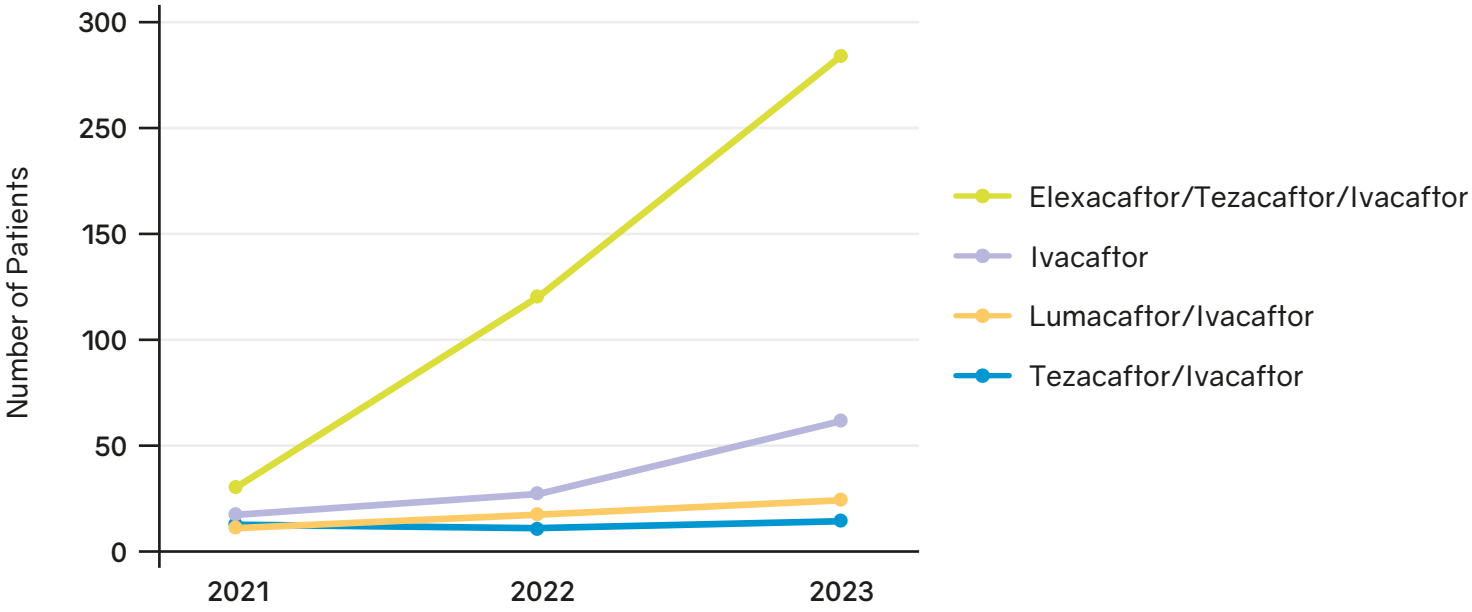
Distribution of therapies



Number of patients using modulator therapy

Modulator treatment	Number of patients
Ivacaftor	55
Lumacaftor/ Ivacaftor	17
Tezacaftor/ Ivacaftor	7
Elexacaftor/ Tezacaftor/ Ivacaftor	279

Patients using CFTR modulators according to years

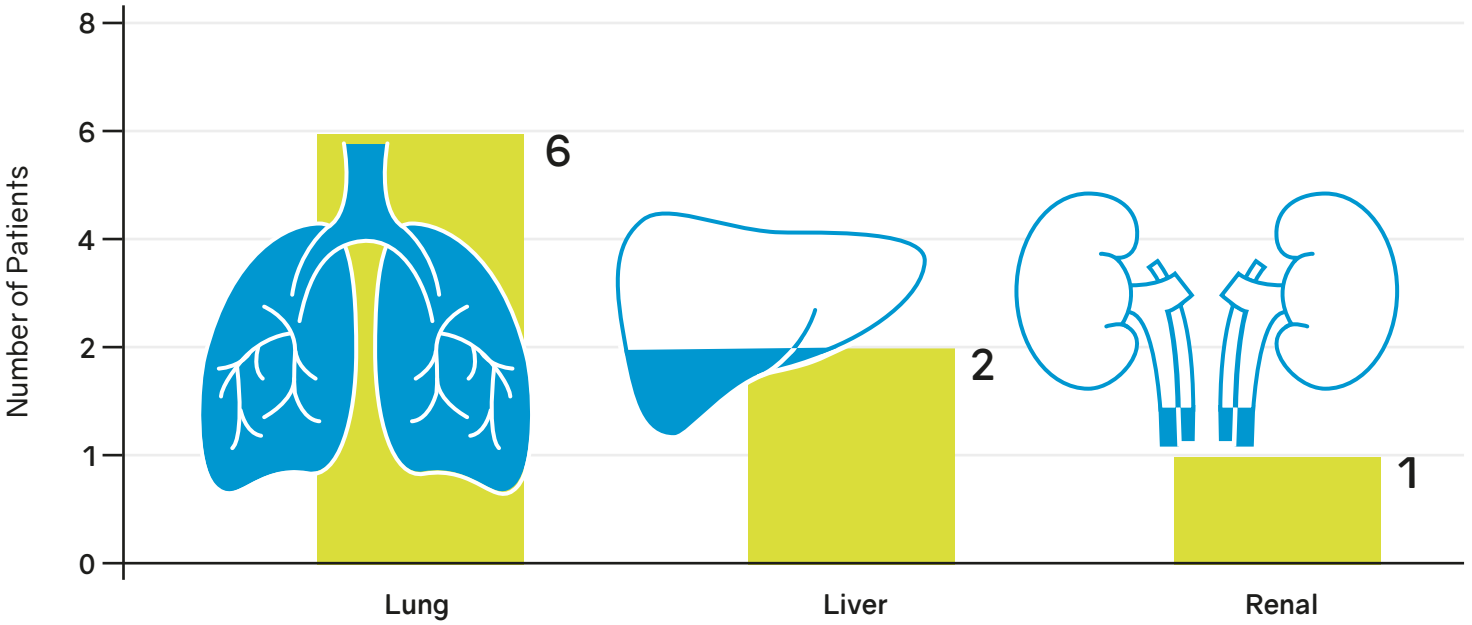


The number of patients using CFTR modulators has been increasing over the years.

Transplantation

- Lung transplantation is recommended in individuals with CF who have progressive respiratory failure and end-stage lung disease.
- Liver transplantation is performed in patients who develop chronic liver failure in CF.

The number of patients with CF living with an organ transplant in 2023



Total: 9