The Belgian Cystic Fibrosis Registry

sciensano

Facts and highlights

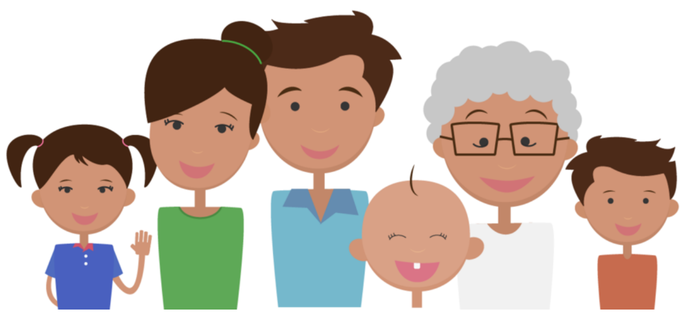
2018 BCFR

Objectives

The aims of the CF registry are to : 1) study disease epidemiology among people with CF in Belgium, 2) provide a tool for the assessment of disease management and quality of care for patients with CF, 3) provide a database for scientific research to CF researchers and 4) to participate in international CF related studies and projects.

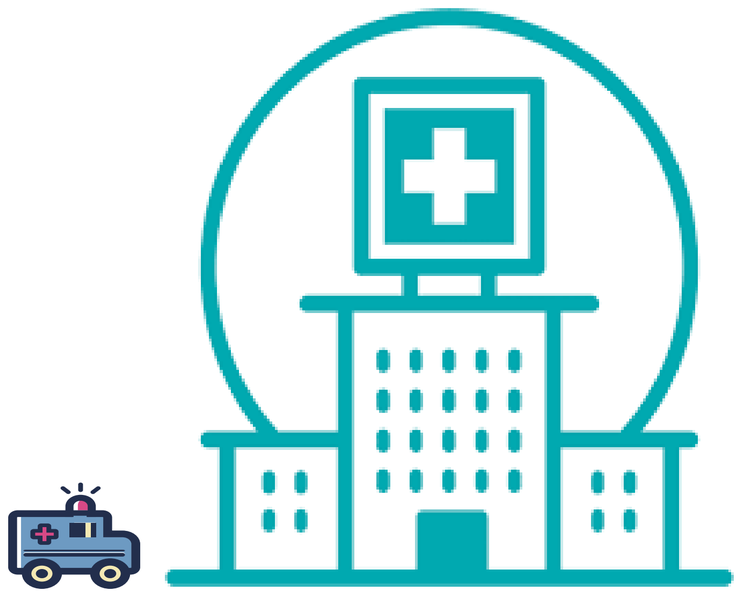
Demographics

There were 1320 People With Cystic Fibrosis (CF) included in the CF registry.



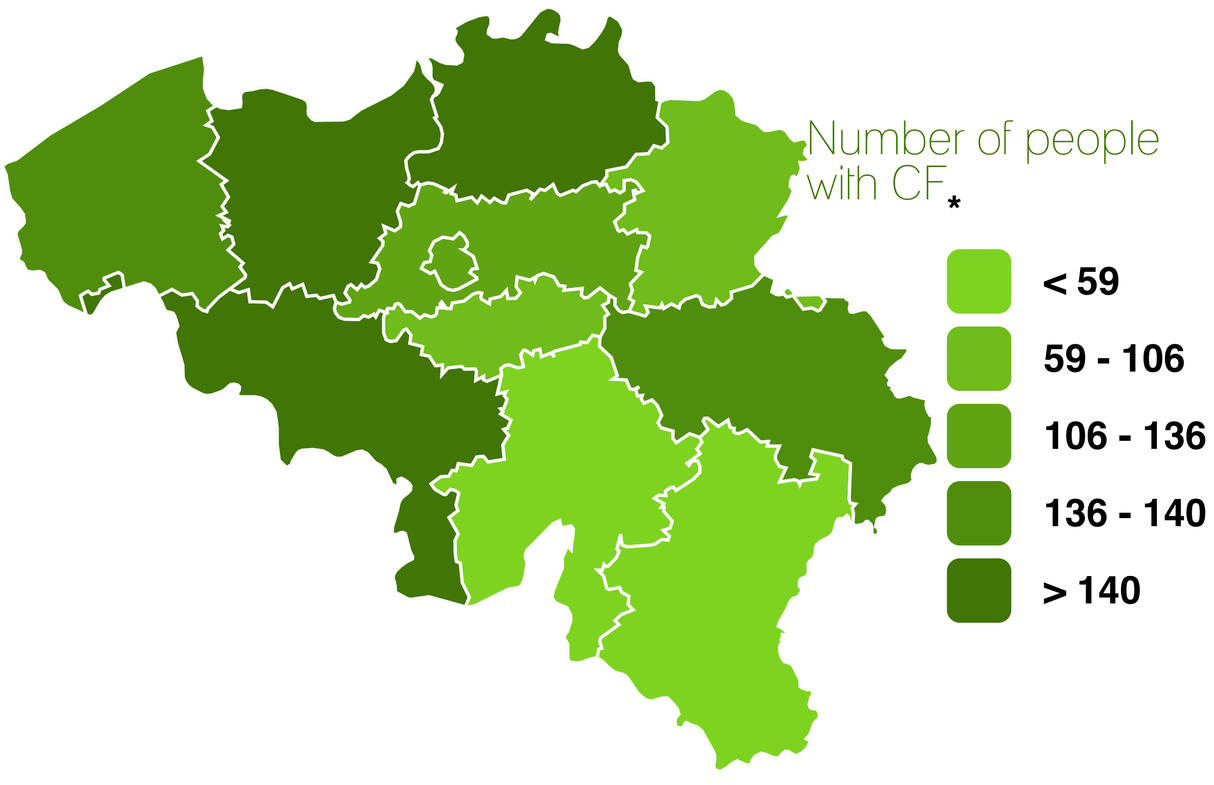
51.9% of the patients are male, 48.1% are female.

There are 7 accredited CF reference centers distributed across the country in 10 clinics, most of them University Hospitals.



Each CF reference center has a pediatric and adult clinic.

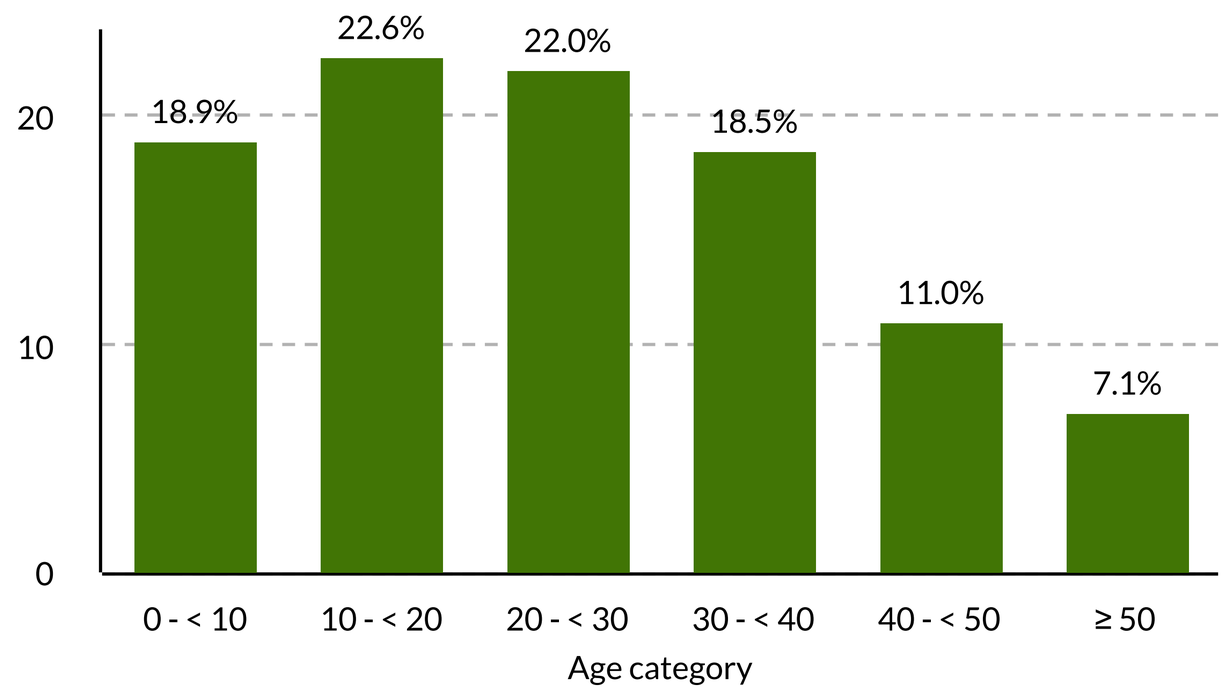
The registry covers about 90 - 95% of CF patients living in Belgium.



* About 15 patients resided outside the country in 2018

The proportion of adults has increased from 38.4% in 2000 to 64.3% in 2018.

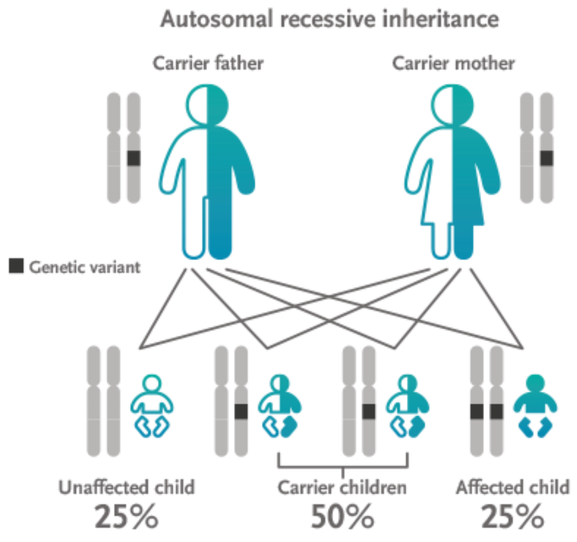
Age distribution in 2018



The median patient age is 23.6 years, (23.0 for male and 23.9 for female patients).

Diagnosis information

A person is born with Cystic Fibrosis (CF) by inheriting a defective copy of the CFTR (CF transmembrane conductance regulator) gene from each parent.



https://www.invitae.com/en/patients/reproductive-health/

The median age at diagnosis is 5.7 months in male and 5.5 months in female patients. There were 22 newly diagnosed CF patients in 2018. Five of the newly diagnosed patients were adults aged 18 years or above. By age 5.0 years, 81.1% of all patients in the registry had been diagnosed.

median age at diagnosis

5.5

Months

Almost half (44.9%) of the patients in the Belgian CF registry are F508del homozygous, 39.6% heterozygous, 13.9% have other mutations while for 0.7% the mutations were not identified.

More than 2000 mutations in the CFTR gene have been identified to date. A majority of these are extremely rare. Not all CFTR mutations lead to CF, and only 346 have been confirmed as disease causing. About 85.0% of the patients seen in 2018 have both mutations in this recent list.

CFTR2_11March2019

https://www.cftrscience.com/cftr-mutations https://www.cftr2.org/

Number of patients and mutation classification by disease liability

Mutation 2

Mutation 1	DC	VCC	UCS	NONCF	NI *	TOTAL
DC	1120	53	89	6	25	1293
VCC		5	2		1	8
UCS			6			6
NONCF		1	1	1	1	4
NI *					9	9
TOTAL	1120	59	98	7	36	1320

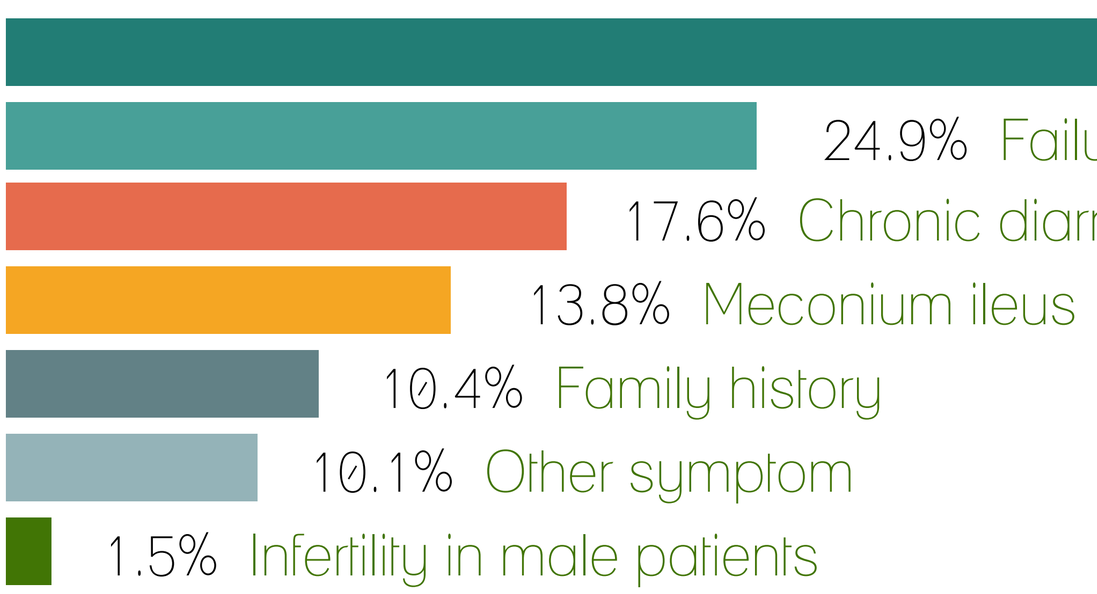
DC = Disease Causing, VCC = Varying Clinical Consequence,
UCS = Unknown Clinical Significance, NONCF= NONCF-Causing,
NI* = Not Identified + Missing data on mutation

The most prevalent mutations are F508del (86.8%), G542X (5.1%), N1303K (4.5%), 3272-26A->G (3.9%) and 1717-1G->A (2.8%)

Homozygous - having two identical alleles of a particular gene
Heterozygous - having two different alleles of a particular gene

Most patients (41.9%) present with acute or recurrent respiratory symptoms. A newborn (neonatal) screening program, coordinated regionally, was introduced in 2019 in Flanders and in January 2020 in Walloonie. A few patients (19.2%) have been diagnosed via local neonatal screening initiatives in the previous years.

Reasons or symptoms at CF diagnosis*



*Not mutually exclusive

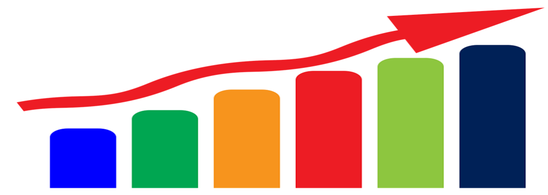
Neonatal screening

19.2%

Definitions :

- The Cystic Fibrosis Transmembrane conductance Regulator (CFTR) is an epithelial ion channel protein that regulates the transport of water and of chloride ions in and out of cells. When the CFTR protein is working correctly, ions freely flow in and out of the cells. However, when the CFTR protein is malfunctioning / defective, these ions cannot flow freely in and out of the cell. This occurs in Cystic Fibrosis, and is characterized by the build-up of a thick, sticky mucus.
- A mutation is a permanent alteration in the DNA sequence that makes up a gene, either due to mistakes when the DNA is copied or as the result of environmental factors, such that the sequence differs from what is found in most people.
- Newborn (neonatal) screening is the practice of testing all babies in their first days of life for certain rare disorders and conditions that can hinder their normal development. Early treatment can improve their health and prevent severe disability or even death.

Registry evolution



Demographics and diagnosis

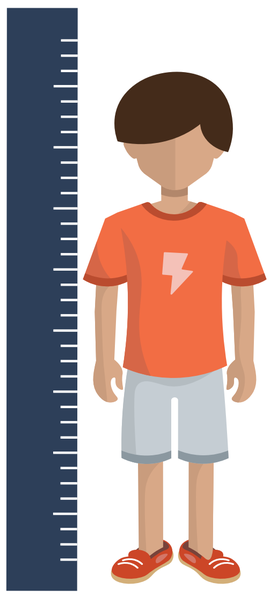
Description of characteristic	2012	2014	2016	2018
Number of CF patients	1189	1230	1279	1332
Patients with complete records	1187	1194	1246	1298
Patients without observation(1)	2	36	33	22
Patients with a transplant	143	153	178	192
Patients who were not seen	17	24	29	35
New CF diagnoses(2)	28	36	23	22
Adults among the newly diagnosed patients	4	6	3	5
Patients without a confirmed diagnosis(3)	50	16	15	11
Revoked diagnosis(4)	25	11	5	3
Median patient age in years (range)(5)	20.3 (0.0 - 71.5)	21.4 (0.1 - 76.9)	22.5 (0.1 - 75.3)	23.6 (0.2 - 77.5)
Median patient age male (range)	20.2 (0.1 - 65.2)	21.3 (0.2 - 67.3)	22.6 (0.5 - 69.2)	23.0 (0.2 - 65.4)
Median patient age female (range)	20.4 (0.0 - 71.5)	21.5 (0.1 - 76.9)	22.5 (0.1 - 75.3)	23.9 (0.7 - 77.5)
Males (%)	612 (51.5%)	639 (52.0%)	664 (51.9%)	691 (51.9%)
Adults ≥ 18 years (%)	673 (56.6%)	720 (58.5%)	784 (61.3%)	856 (64.3%)
Median age at diagnosis (months) (6)	5.6	5.6	5.4	5.5
Age range at diagnosis (years)	-0.2 - 65.0	-0.5 - 74.2	-0.5 - 65.2	-0.2 - 70.6
Median age at diagnosis, male (months)	5.3	5.7	5.8	5.7
Age range at diagnosis, male (years)	-0.2 - 46.9	-0.5 - 59.5	-0.5 - 59.5	-0.2 - 59.5
Median age at diagnosis, female (months)	5.7	5.6	5.2	5.5
Age range at diagnosis, female (years)	-0.2 - 65.0	-0.2 - 74.2	-0.2 - 65.2	-0.1 - 70.6
Median age at diagnosis for new cases, months (range)	1.8 (-1.8 - 430.0)	2.5 (0.0 - 720.1)	3.8 (-0.9 - 627.4)	9.8 (0.1 - 846.6)
Median age at diagnosis new cases in years (range)	0.1 (-0.2 - 35.8)	0.2 (0.0 - 60.0)	0.3 (-0.1 - 52.3)	0.8 (0.0 - 70.6)
Number of transplants performed	13	11	20	16
Total number of deaths reported	12	10	8	16
Median age at death in years (range)	32.3 (8.9 - 52.3)	37.3 (11.5 - 76.9)	31.3 (20.5 - 44.8)	44.1 (20.4 - 70.2)
Deaths among transplant patients	5	4	4	9

1. Patients without at least four filled-in clinical items, postulated alive or registered as deceased in the collection year, are not used in the analysis of clinical data. Patients may be postulated alive or registered as deceased within the year but have missing clinical data
2. The new CF diagnoses are patients with the earliest diagnosis date from amongst the clinical diagnosis date, TEPD date, genotype date or the sweat test date done within the data registration year.
3. Patients without a confirmed diagnosis are not included in the total number of CF patients
4. Patients with a revoked diagnosis are not included in the total number of CF patients
5. Patient's age at the last consultation
6. Prenatal diagnosis is considered without setting to zero the age at diagnosis allowing negative values

Growth and nutrition

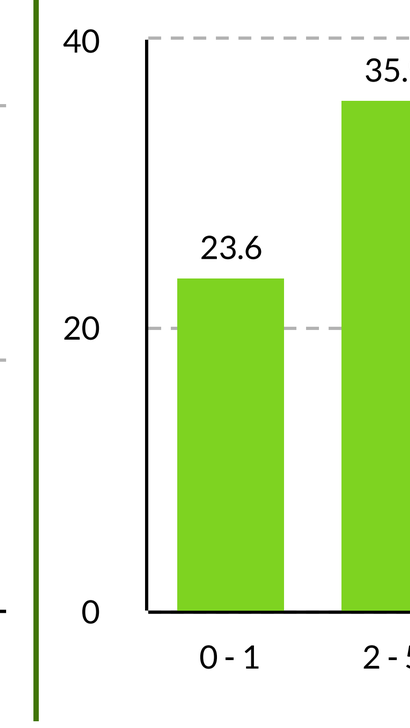
The nutritional status (median BMI) of CF patients in Belgium has improved in the last 2 decades, most importantly in adolescents and young adults.The goal of a good nutritional status is to have a median percentile of 50 or above for the BMI, height and weight.

With CF, patients may have stunted growth and may not grow tall as quickly as peers of the same age and gender.



Median Height percentile in patients 0 - 20 years

34.7



Weight gain is always a challenge in CF. A well balanced high calorie, high protein and high fat diet as well as the intake of pancreatic enzymes are crucial to achieving a good nutritional status.

Median Weight percentile in patients 0 - 20 years

32.3

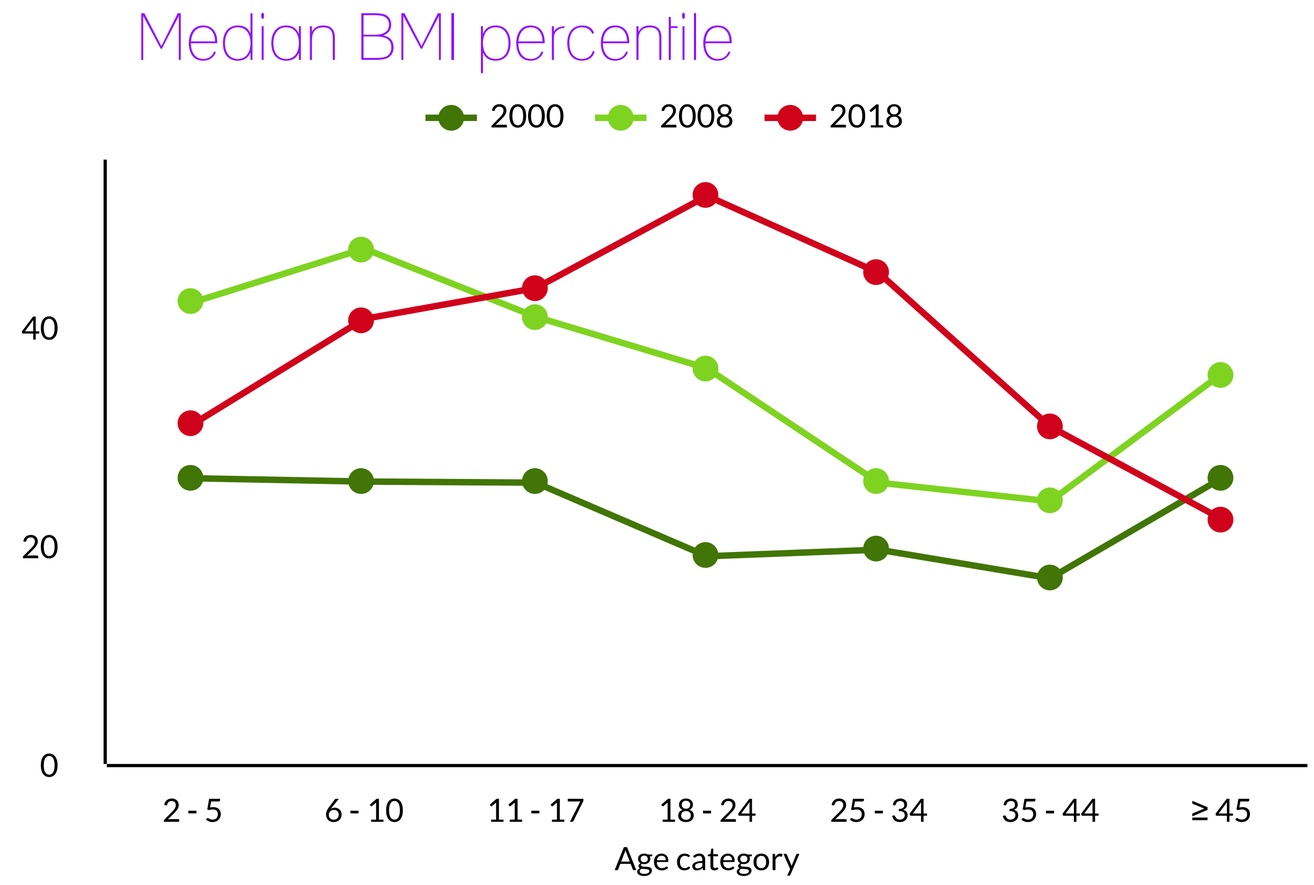
Nutritional care is of great importance for patients with CF. Because of thick mucus, the pancreas is unable to produce and/or carry digestive enzymes to the gut. This leads to poor absorption of proteins, fats and fat soluble vitamins resulting in poor weight gain and growth. Maintaining or achieving a better nutritional status has a positive impact on lung function.

Adult patients with BMI between 18.5kg/m^2 and 25kg/m^2

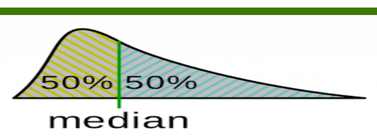
69.9%

Median BMI percentile in patients 2 - 20 years

36.1



Definitions :



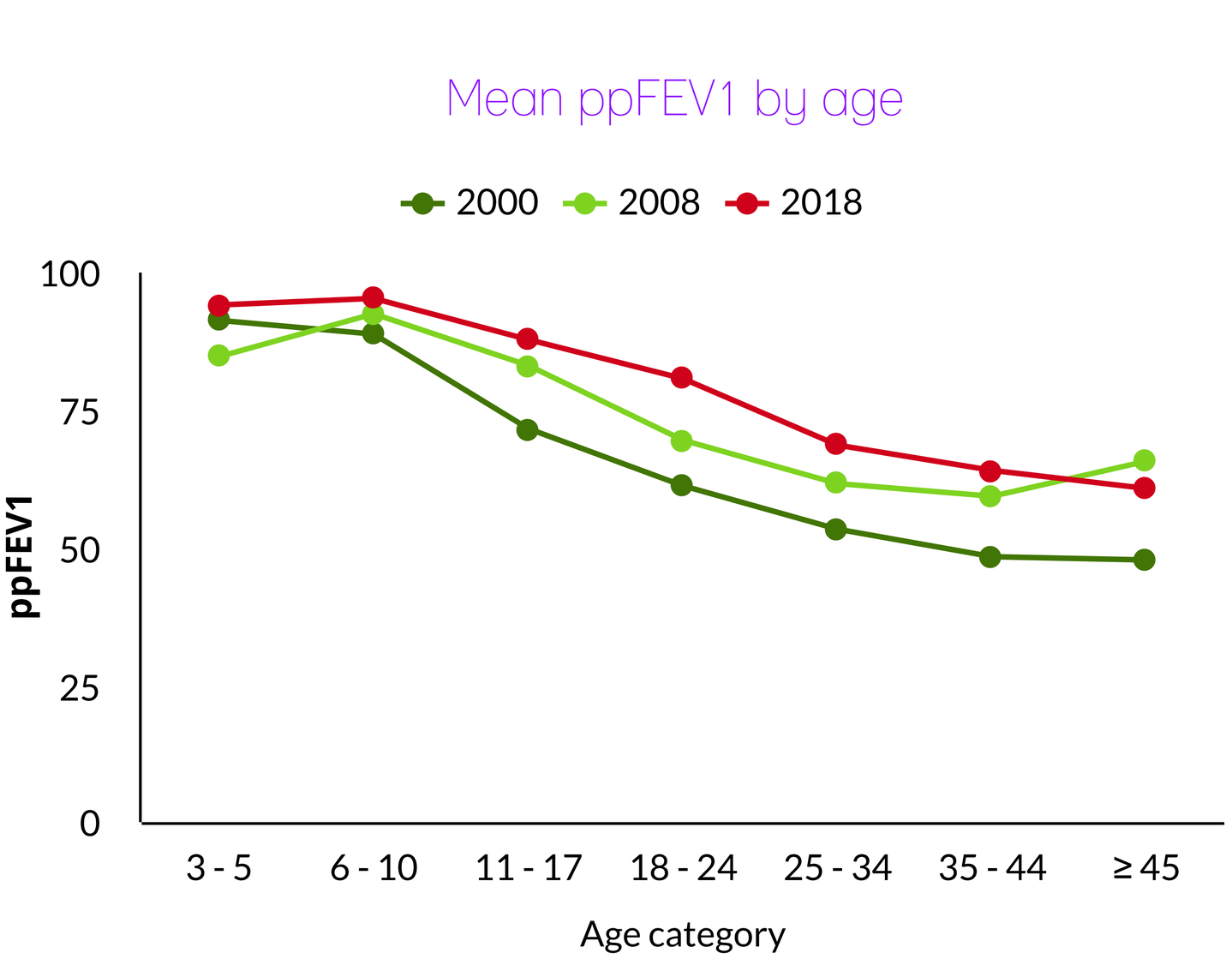
- A median is a point that divides the data into two numerically equal groups based on their ordered values. Median age implies that half the people are younger than this age and half are older.
- A percentile is each of the 100 equal portions into which a group of values can be divided according to the distribution of these values. The median is also the 50th percentile (P50) and is the value at which half of the observations are larger and the other half smaller. The "mean", also called the "average", is the sum of all the elements in a group then divide this sum by the number of elements used.
- BMI is the ratio between a person's weight (in kg) and the height (expressed in m^2).

Lung function

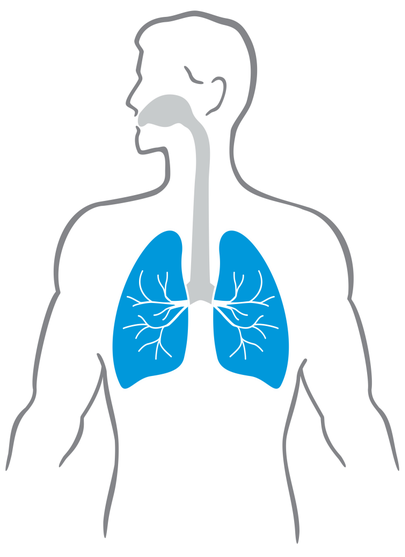
In the last 2 decades, there have been improvements in lung function across most age categories.

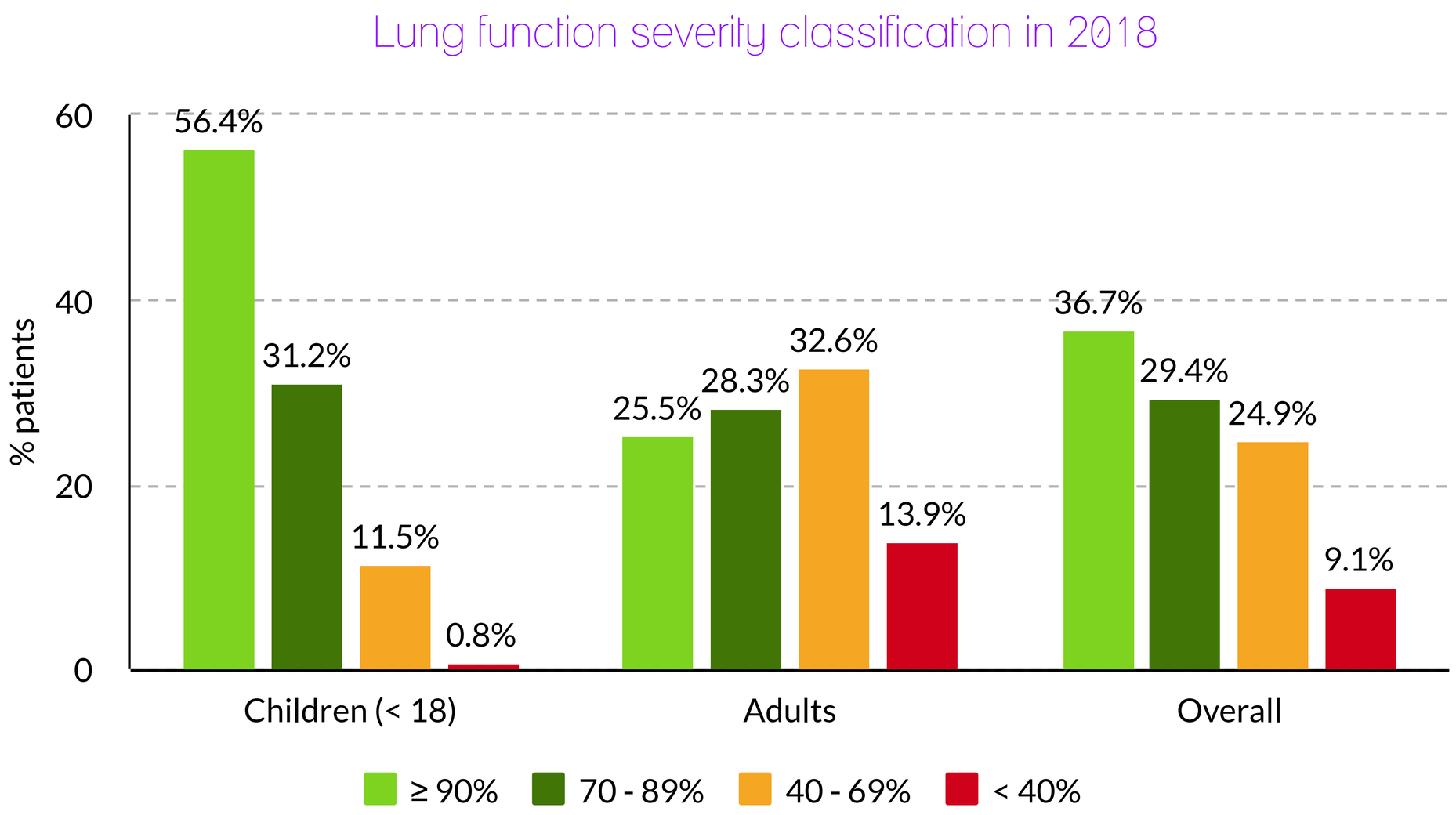
The percentage of predicted forced expiratory volume in one second (ppFEV1) is a clinical parameter used to monitor lung function impairment.
Because most patients with CF develop progressive pulmonary disease, measures of pulmonary involvement, in particular ppFEV1, are used to follow up the lung disease.
The ppFEV1 partly determines the prognosis. However, considerable heterogeneity exists in prognosis and disease severity, even among patients with the same mutations.

Schluchter MD, 2006, McKone E, 2003



FEV1% predicted values (ppFEV1), based on Global Lung Initiative (GLI) references, were divided in four classes corresponding to different degrees of lung function impairment: near normal (≥ 90%), mild impairment (70 - 89%), moderate impairment (40 - 69%) and severe impairment (< 40%).



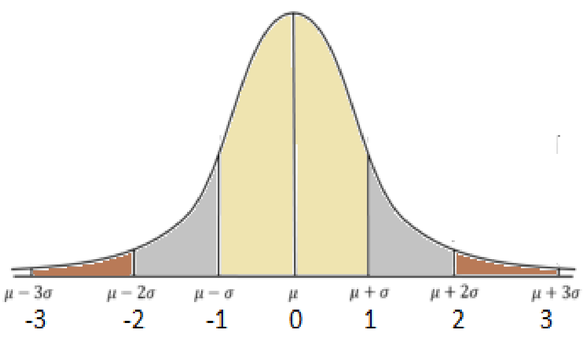


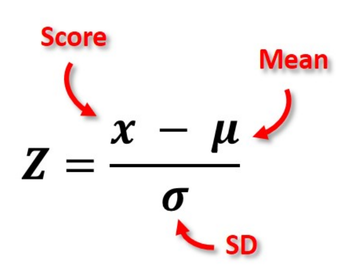
Definitions :

- The forced expiratory volume in one second (FEV1) is the amount of air that a person is able to exhale forcefully in one second, following full inspiration. It is expressed as a percentage of the predicted value (ppFEV1) for a reference population with same age, gender, height and ethnic background.
- The forced vital capacity (FVC) is the total volume of air a patient is able to exhale for the total duration of the test during maximal effort, expressed also as a percentage of the predicted value.
- A z-score describes the position of a raw score (x) in terms of its distance from the mean (μ), when measured in standard deviation units (σ). If a Z-score is 0, it indicates that the data point's score is identical to the mean score. The z-score is positive if the value lies above the mean, and negative if it lies below the mean.

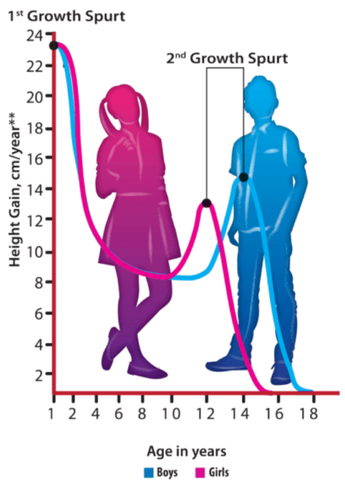
Registry evolution

Description of characteristic (7)	2012	2014	2016	2018
SPIROMETRY (FEV1)				
FEV1 % predicted, Last of year- Mean (SD)	76.2 (24.0)	75.7 (24.6)	76.0 (23.8)	78.1 (24.6)
Male	76.9 (23.8)	76.5 (24.4)	77.1 (23.2)	79.2 (24.2)
Female	75.4 (24.3)	74.9 (24.8)	74.8 (24.4)	76.9 (25.0)
Children	87.0 (20.3)	88.3 (19.7)	87.9 (18.3)	91.1 (18.5)
Adults	67.4 (23.3)	67.0 (23.9)	68.3 (23.8)	70.5 (24.5)
FEV1 % predicted, Best of year - Mean (SD)	80.5 (23.1)	80.2 (23.3)	80.5 (23.4)	82.3 (23.8)
Male	81.0 (22.9)	81.0 (22.9)	81.2 (22.8)	83.3 (23.4)
Female	79.9 (23.3)	79.3 (23.7)	79.8 (24.0)	81.2 (24.2)
Children	91.7 (18.1)	93.0 (17.0)	93.1 (17.3)	96.4 (17.1)
Adults	71.3 (22.6)	71.3 (22.9)	72.3 (23.1)	74.1 (23.3)
FEV1 % predicted z-score, Last of year - Mean (SD)	-1.9 (1.9)	-1.9 (1.9)	-1.9 (1.9)	-1.7 (1.9)
Male	-1.8 (1.9)	-1.9 (1.9)	-1.8 (1.8)	-1.6 (1.9)
Female	-2.0 (1.9)	-2.0 (2.0)	-2.0 (1.9)	-1.8 (1.9)
Children	-1.1 (1.7)	-1.0 (1.6)	-1.0 (1.5)	-0.7 (1.5)
Adults	-2.6 (1.8)	-2.6 (1.9)	-2.5 (1.8)	-2.3 (1.9)
FEV1 % predicted z-score, Best of year - Mean (SD)	-1.6 (1.8)	-1.6 (1.8)	-1.5 (1.8)	-1.4 (1.9)
Male	-1.5 (1.8)	-1.5 (1.8)	-1.5 (1.8)	-1.3 (1.8)
Female	-1.6 (1.9)	-1.6 (1.9)	-1.6 (1.9)	-1.5 (1.9)
Children	-0.7 (1.5)	-0.6 (1.4)	-0.6 (1.4)	-0.3 (1.4)
Adults	-2.3 (1.8)	-2.3 (1.8)	-2.2 (1.8)	-2.0 (1.8)
ANTROPOMETRY (BMI, HEIGHT AND WEIGHT)				
BMI Z-score (last of year), median (range)	-0.4 (-4.1 - 2.8)	-0.4 (-3.9 - 2.3)	-0.4 (-4.0 - 2.5)	-0.4 (-3.3 - 2.6)
Weight Z-score (last of year), median (range)	-0.6 (-6.9 - 2.3)	-0.5 (-4.2 - 2.6)	-0.5 (-4.1 - 2.6)	-0.5 (-4.7 - 2.7)
Height Z-score (last of year), median (range)	-0.4 (-4.1 - 3.2)	-0.4 (-4.1 - 3.2)	-0.3 (-3.9 - 2.7)	-0.4 (-3.5 - 2.6)





$$Z = \frac{x - \mu}{\sigma}$$

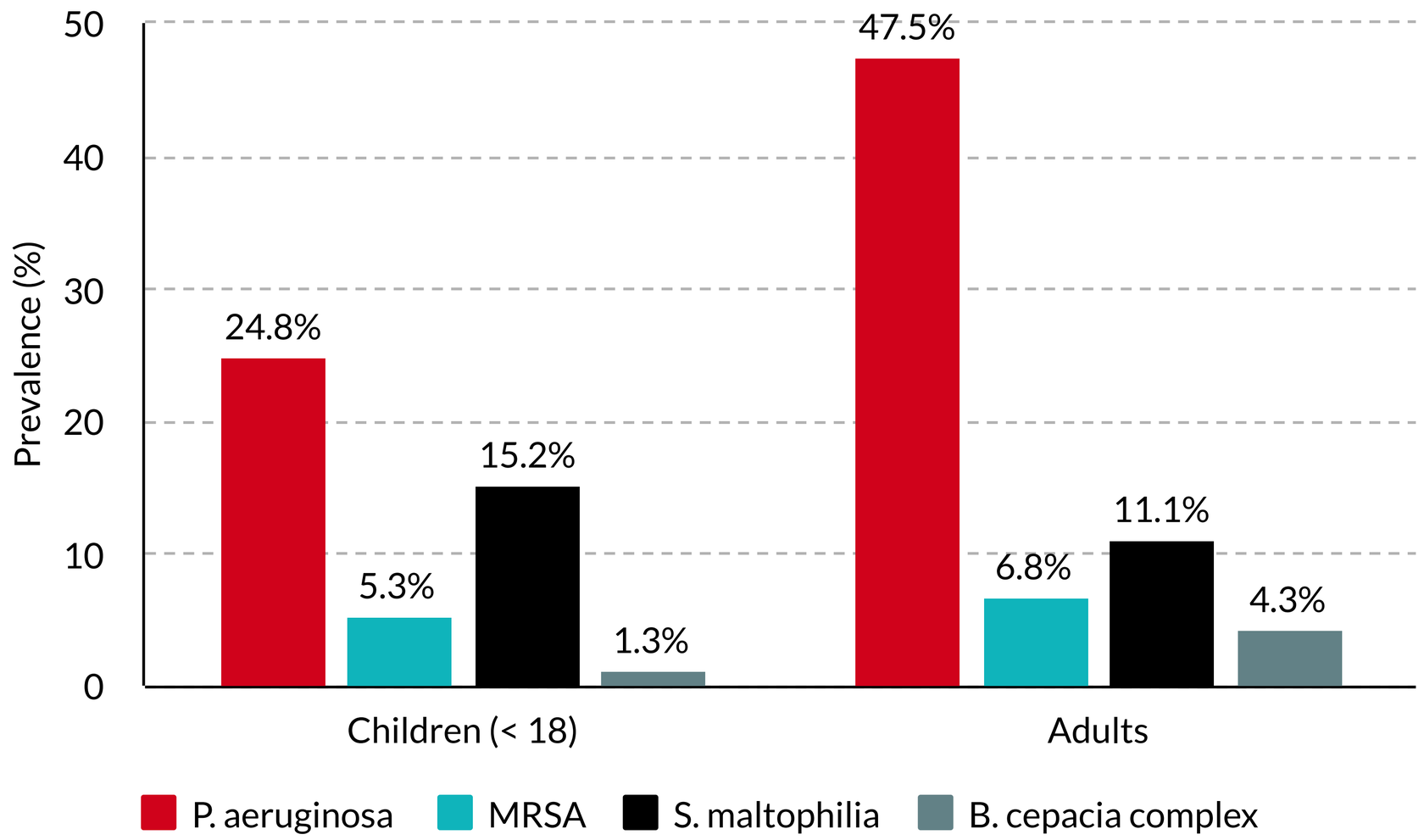


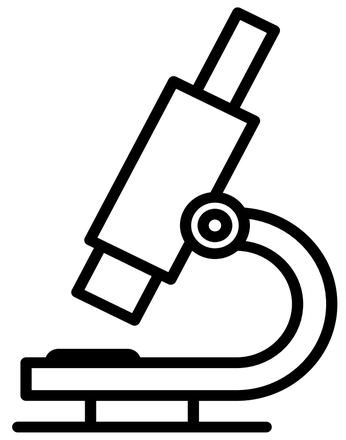
7. Transplant patients are excluded from the analysis of spirometry and anthropometry data
   The analysis of spirometry data is for patients aged 3 years and above and is based on Global Lung Initiative reference equations.

Microbiology

One of the main goals of CF care is to prevent or postpone infections and to reduce the risk of chronic infections (or colonization), which increases respiratory morbidity and treatment burden.

Prevalence of infections in 2018





MRSA - Methicillin Resistant Staphylococcus aureus

Sputum samples, throat swabs and bronchoalveolar lavage cultures are taken to monitor the presence of pathogens. At least 82.6% of the patients had 4 or more cultures taken in the year 2018.

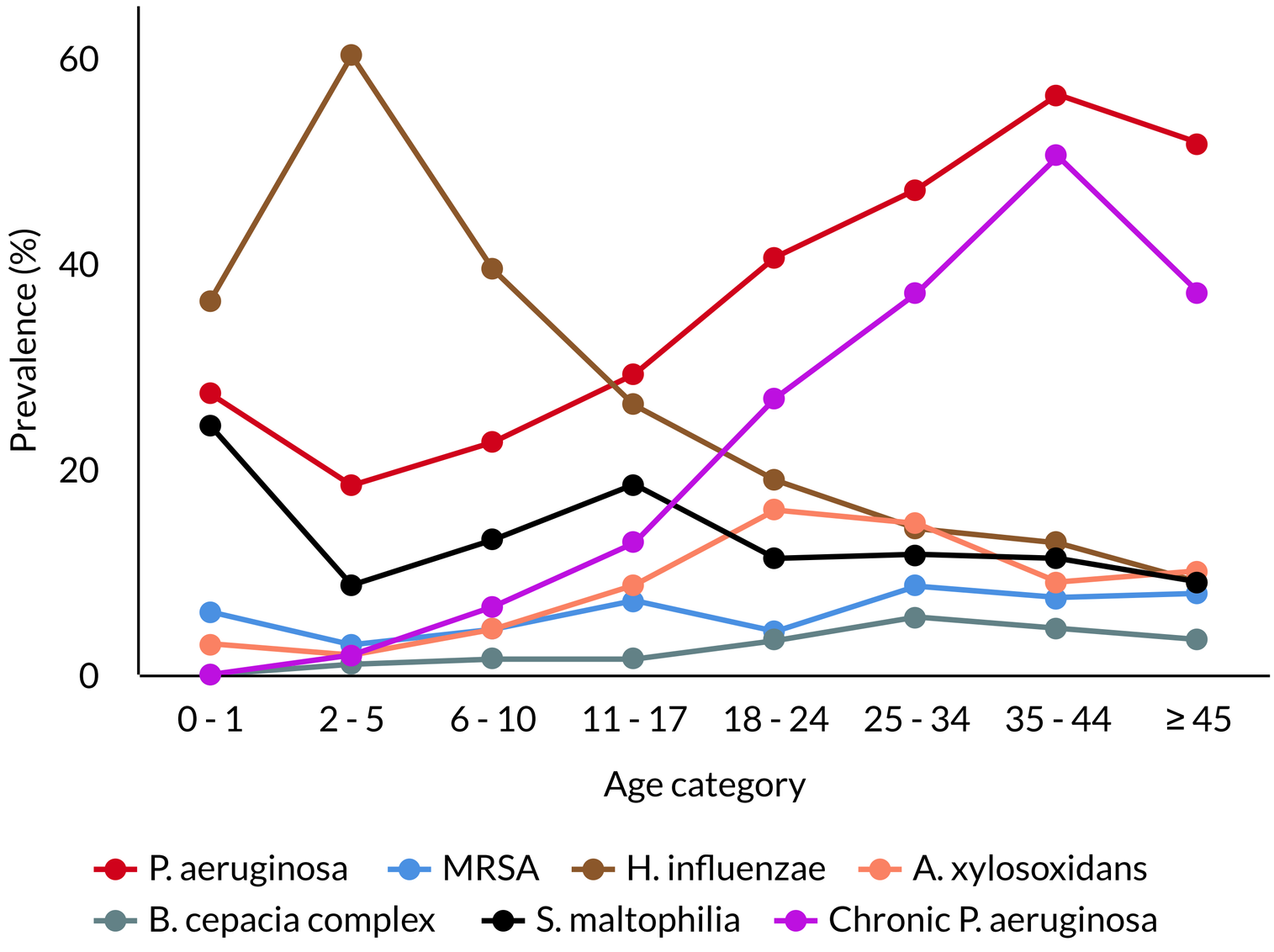
Bacterial infection may occur very early in the natural history of the disease. In children common bacteria such as Staphylococcus aureus and Haemophilus influenzae may infect the lungs.

Infection by Pseudomonas aeruginosa and sometimes Burkholderia cepacia complex and other gram-negative pathogens occur at a later age. The airways of patients with CF may also be chronically colonized by fungi like Aspergillus fumigatus.

Hart CA 2002, de Vrankrijker AM, 2011

The figure shows a high prevalence of H. influenzae at a very young age, with its reduction with age replaced by the progressive increase of the other bacteria especially P. aeruginosa.

Age related prevalence of microbes in 2018



Definitions :

- Prevalence is the proportion of a population that is affected or that has a given attribute at a given time.
- Chronic Pseudomonas aeruginosa infection is defined as having more the 50% of the sputum samples positive taken in a 12 months period with at least 4 sputum samples taken during that period.

Complications

Cystic Fibrosis affects the respiratory, digestive, and reproductive systems with variable degrees of severity.

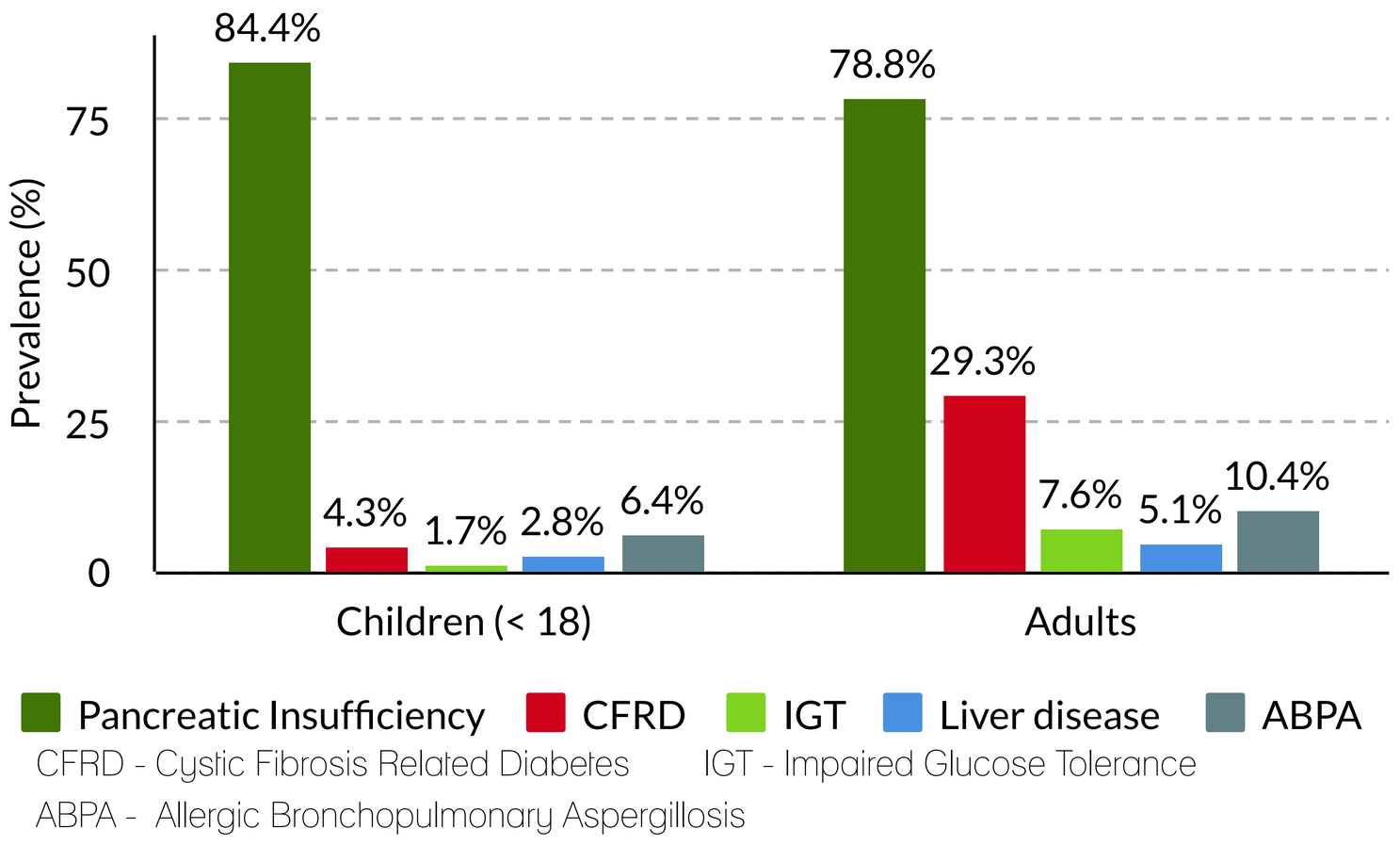
The defective chloride channel in CF causes a range of disturbances within the human body.

In CF, the transport through the cell wall of chloride, other ions and water are disturbed.

Complications in CF are mainly found in organs where mucus linings are needed (airways, intestines) and in glands which need fluid to excrete their substances (pancreas, testicles…).

Flume PA 2009, Sinaasappel M 2002, Goodin B 2005, McCallum TJ 2000, Lyon A 2002

Prevalence of complications in 2018



CFRD - Cystic Fibrosis Related Diabetes IGT - Impaired Glucose Tolerance

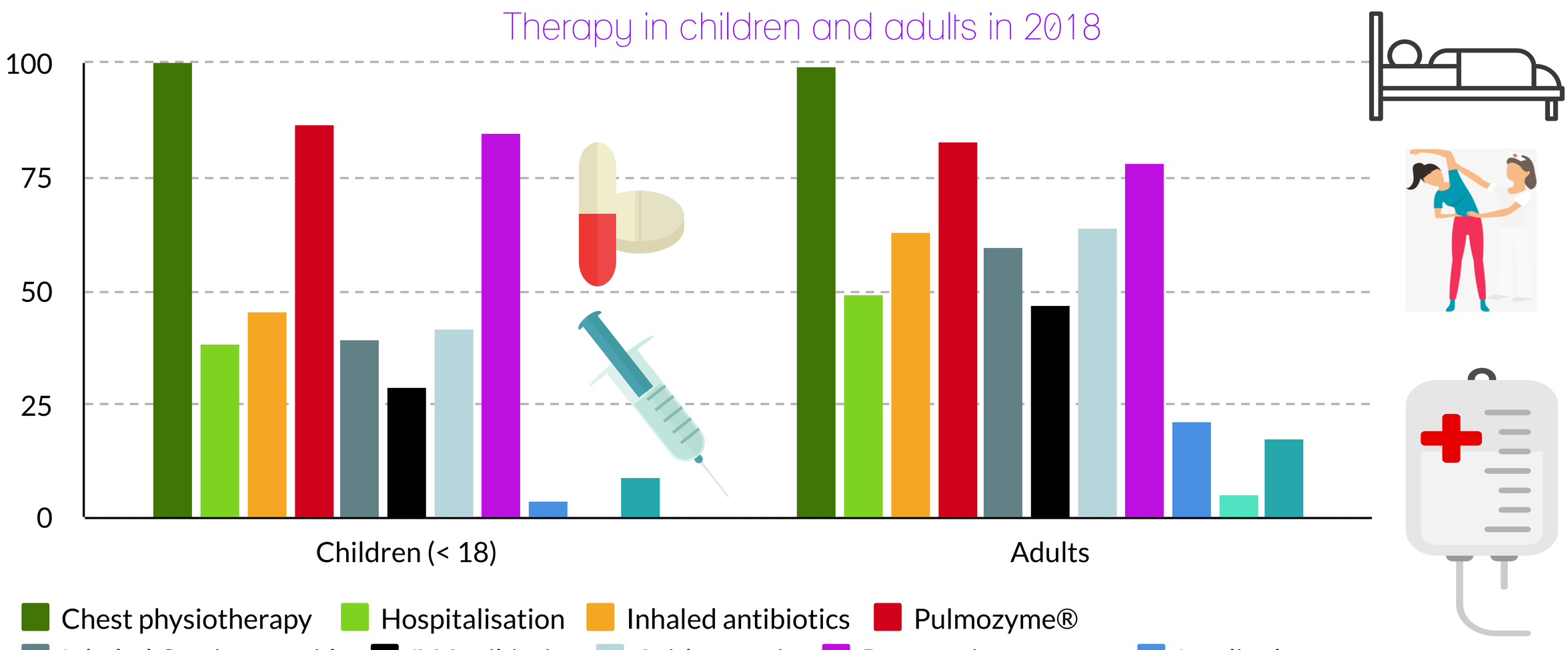
ABPA - Allergic Bronchopulmonary Aspergillosis

The lower prevalence of Pancreatic Insufficiency in adults is due to a higher number of patients with mild genotype and Pancreatic Sufficiency in the adult patients. The most important other complication is CFRD, especially among adults.

Treatment burden

There is currently no definitive cure for CF. Treatment of CF remains mostly based on preventing or reducing symptoms in order to avoid complications or to stabilize them. There are now new therapies in the pipeline that target the basic defect.
During the year 2018, 82.6% of the patients had at least 4 required visits to the clinic.

Therapy in children and adults in 2018



Definitions :

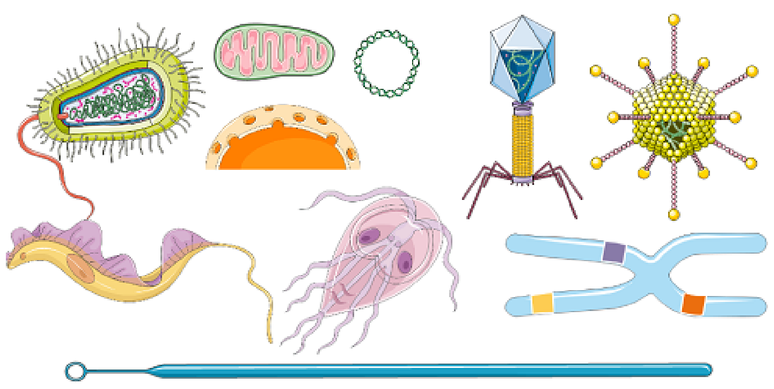
- ABPA is an allergic reaction to Aspergillus fumigatus a fungus that colonizes the airways of some people with CF.
- CFRD / IGT - The malfunctioning of the endocrine part of the pancreas by fibrosis leads to an insufficient secretion of insulin leading to IGT or diabetes.
- Exocrine pancreatic insufficiency (PI) is the inability of the pancreas to produce and transport enough pancreatic enzymes to the duodenum to digest fat and proteins resulting in malabsorption with steatorhea (fatty stools), malnutrition and a deficiency in fat-soluble vitamins.
- Liver disease - Due to a defective CFTR protein some patients have problems in their biliary tract, where bile is thick and sticky and has difficulty getting out of the liver, causing irritation and inflammation in the bile ducts and leading to scarring (cirrhosis).

Registry evolution

Description of characteristic	2012	2014	2016	2018
MICROBIOLOGY (8)				
Pseudomonas aeruginosa	421 (42.3%)	427 (41.2%)	399 (37.5%)	416 (37.9%)
Burkholderia cepacia complex	40 (4.0%)	38 (3.7%)	36 (3.4%)	33 (3.0%)
Methicillin Resistant Staphylococcus aureus (MRSA)	88 (8.8%)	76 (7.3%)	57 (5.4%)	68 (6.2%)
Haemophilus influenzae	297 (29.8%)	279 (26.9%)	249 (23.4%)	272 (24.7%)
Stenotrophomonas maltophilia	118 (11.8%)	128 (12.4%)	142 (13.3%)	141 (12.8%)
Achromobacter xylosoxidans	106 (10.6%)	102 (9.8%)	110 (10.3%)	110 (10.0%)
Aspergillus spp.	346 (34.7%)	364 (35.1%)	364 (34.2%)	370 (33.7%)
Non-tuberculous mycobacterium (NTM)	-	11 (1.1%)	18 (1.7%)	17 (1.5%)
CHRONIC INFECTIONS				
Chronic *Pseudomonas aeruginosa*	276 (27.7%)	290 (28.0%)	273 (25.7%)	266 (24.2%)
Chronic *Burkholderia cepacia* complex	26 (2.6%)	34 (3.3%)	27 (2.5%)	24 (2.2%)
Chronic *Stenotrophomonas maltophilia*	27 (2.7%)	40 (3.9%)	40 (3.8%)	44 (4.0%)
Chronic *Achromobacter xylosoxidans*	54 (5.4%)	66 (6.4%)	72 (6.8%)	69 (6.3%)
Chronic *MRSA*	51 (5.1%)	43 (4.2%)	40 (3.8%)	47 (4.3%)
COMPLICATIONS				
Allergic Bronchopulmonary Aspergillosis (ABPA)	41 (3.9%)	72 (6.9%)	68 (6.3%)	97 (8.7%)
Liver disease	37 (3.5%)	42 (3.9%)	66 (6.0%)	46 (4.0%)
Pancreatic Insufficiency	831 (79.1%)	865 (82.5%)	880 (81.9%)	901 (81.0%)
CF related diabetes (CFRD)	136 (13.0%)	181 (17.3%)	185 (17.2%)	208 (18.7%)
Bronchiectasis (9)	-	306 (73.0%)	402 (78.2%)	329 (78.1%)

8. Only patients who had a culture or sample taken were included. Transplanted patients were excluded from the analysis of infections and complications.

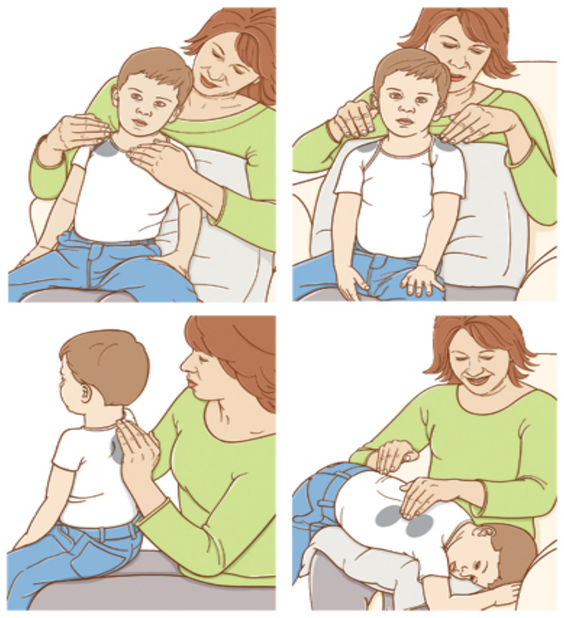
9. Only patients who had a CT scan done during the year were considered in the case of Bronchiectasis.



Registry evolution

Therapy, treatment and medication

Description of characteristic (10)	2012	2014	2016	2018
RESPIRATORY THERAPY				
Regular chest physiotherapy	1014 (96.6%)	1044 (99.5%)	1071 (99.7%)	1108 (99.6%)
Oral Antibiotics only	501 (47.7%)	477 (45.5%)	509 (47.4%)	471 (42.4%)
IV Antibiotics only	11 (1.0%)	13 (1.2%)	18 (1.7%)	23 (2.1%)
Oral and IV Antibiotics	400 (38.1%)	401 (38.2%)	404 (37.6%)	414 (37.2%)
RhDnase	732 (69.7%)	806 (76.8%)	892 (83.1%)	941 (84.6%)
Other mucolytics	238 (22.7%)	215 (20.5%)	184 (17.1%)	152 (13.7%)
Hypertonic saline	576 (54.9%)	630 (60.1%)	675 (62.8%)	745 (67.0%)
Inhaled corticosteroids	576 (54.9%)	547 (52.1%)	578 (53.8%)	568 (51.1%)
Bronchodilators	778 (74.1%)	818 (78.0%)	811 (75.5%)	862 (77.5%)
Intranasal steroids	459 (43.7%)	535 (51.0%)	533 (49.6%)	589 (53.0%)
Oral Azithromycin	475 (45.2%)	497 (47.4%)	558 (52.0%)	607 (54.6%)
Oral Systemic Corticosteroids	58 (5.5%)	58 (5.5%)	33 (3.1%)	54 (4.9%)
Oral Nonsteroidal anti-inflammatory drugs (NSAIDs)	44 (4.2%)	63 (6.0%)	37 (3.4%)	32 (2.9%)
Oxygen Therapy	21 (2.0%)	35 (3.3%)	21 (2.0%)	32 (2.9%)
GASTRO-INTESTINAL AND NUTRITIONAL THERAPY				
Pancreatic enzymes	845 (80.5%)	871 (83.0%)	881 (82.0%)	902 (81.1%)
Fat soluble vitamins (ADEK)	846 (80.6%)	874 (83.3%)	882 (82.1%)	893 (80.3%)
Proton pump Inhibitors and/ or H2 blockers	452 (43.0%)	513 (48.9%)	540 (50.3%)	543 (48.8%)
Gastrotomy tube feeding	32 (3.0%)	38 (3.6%)	46 (4.3%)	45 (4.0%)
OTHER MEDICATION				
Ursodeoxycholic acid	256 (24.4%)	257 (24.5%)	245 (22.8%)	218 (19.6%)
Insulin therapy	108 (10.3%)	128 (12.2%)	139 (12.9%)	154 (13.8%)
Oral Therapy for Diabetes	25 (2.4%)	28 (2.7%)	32 (3.0%)	34 (3.1%)
CFTR Modulating Therapy	-	52 (5.0%)	106 (9.9%)	155 (13.9%)



10. Transplant patients are excluded from the analysis of therapy , treatments and medication.

Transplantation and Cystic Fibrosis

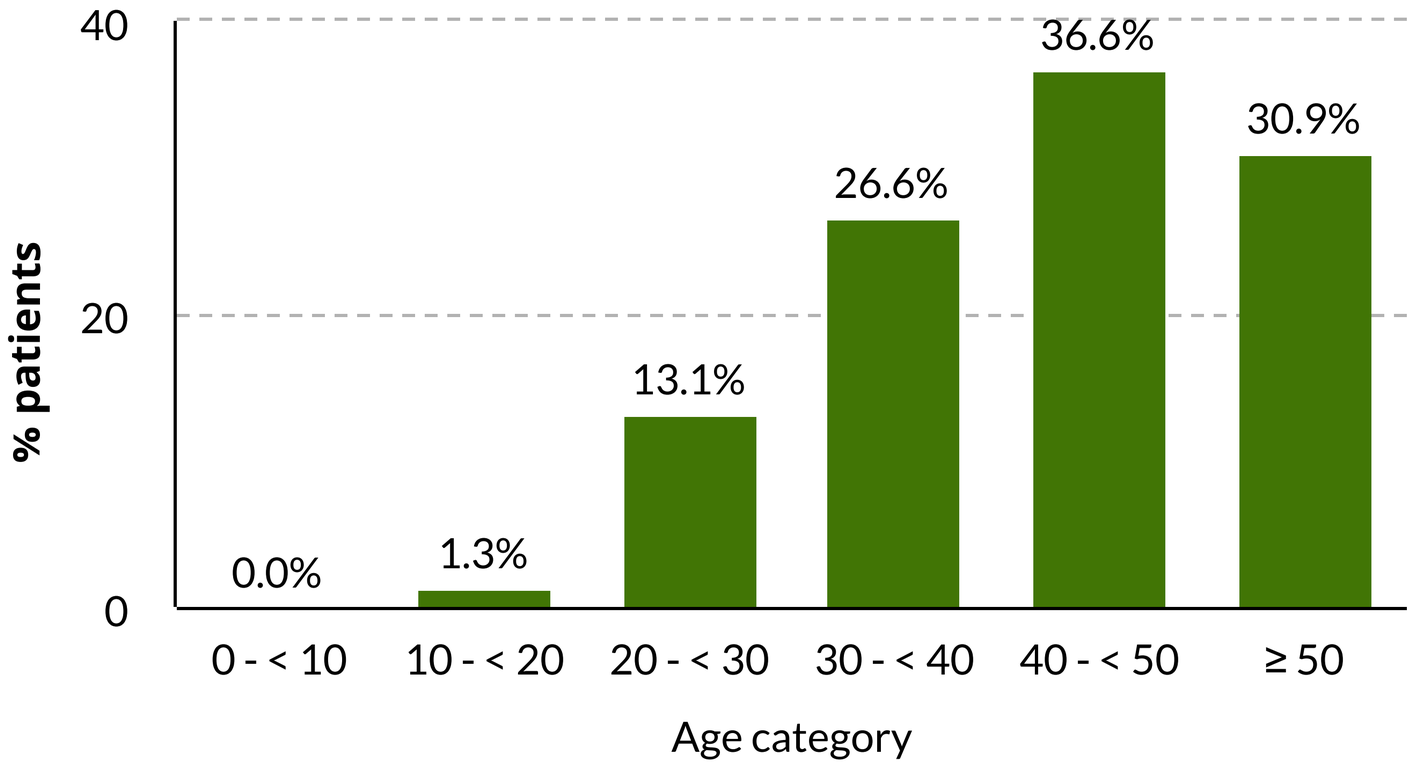
When a patient with CF develops severe and progressive lung disease, lung transplantation may become an option. A lung transplant is indicated only for patients who have a severe disease, who have exhausted all other forms of conventional medical treatment and whose short term survival is compromised.

The registry records show that since its inception in 1999, at least 274 patients, 129 male and 145 female, have benefited from transplantation.

In 2018, there were 189 patients (89 male, 100 female) who were alive after receiving a transplanted organ in the past.

Their median age (range) was 37.7 (14.6 – 69.8) years. 98.4% of these transplant patients were adults.

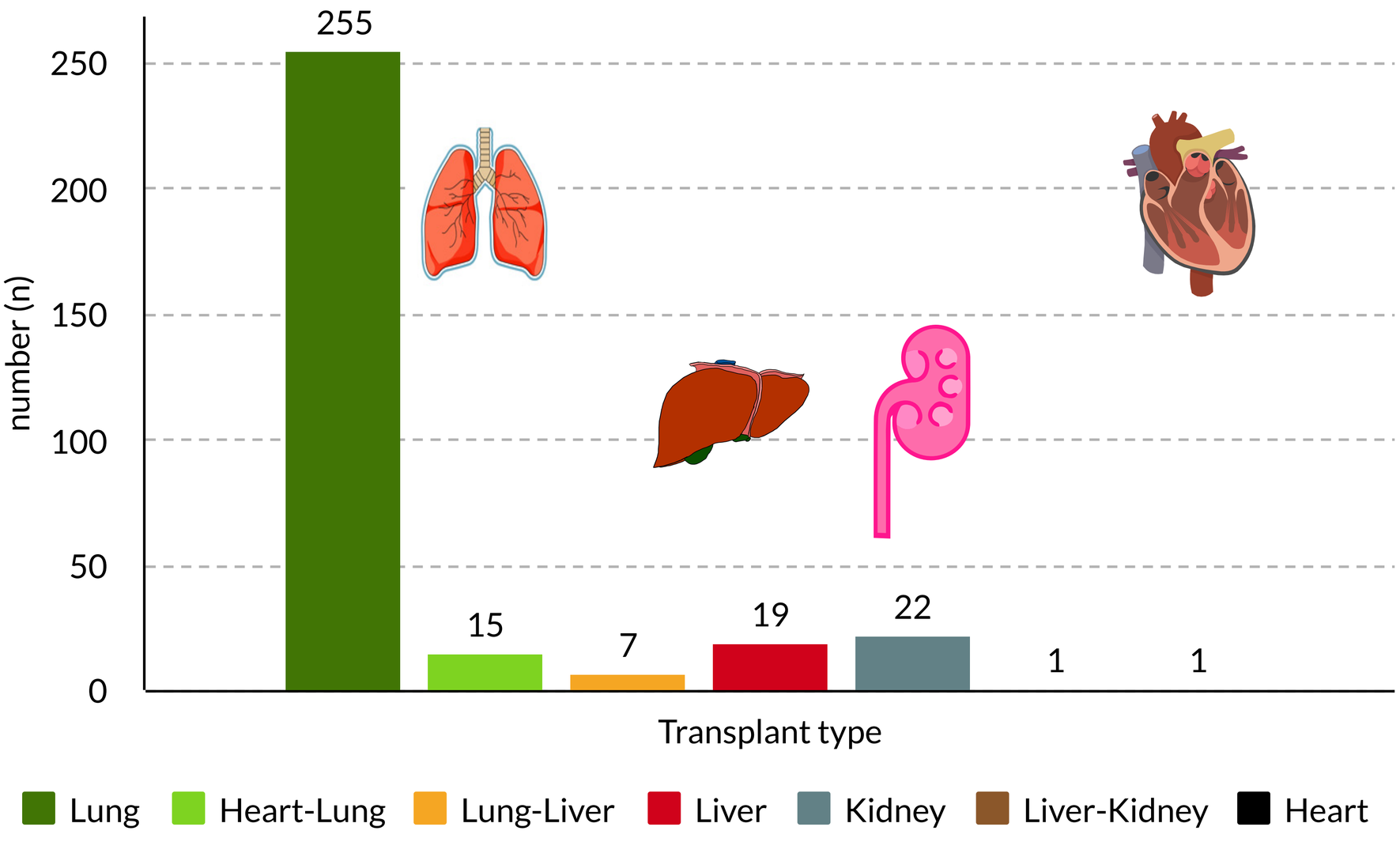
Age distribution of transplanted patients



In 2018 at least one in every five adult patients (22.0%) was a transplant patient.

The most frequent organ transplant done to Cystic Fibrosis patients in Belgium is the lung transplant. A few patients will need other types of organ transplants such as a liver or kidney. About 320 transplants have been reported since the inception of the registry.

Type of transplant



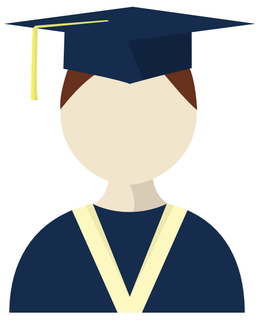
Life statistics

People with CF are living longer. This introduces new challenges and expectations alike. Despite their therapy burden, they are now studying, graduating and taking up a career either part-time or even full-time and some are raising a family. They are thus taking up roles in society that were previously less attainable by cohorts decades back.

In 2018, almost half of the adult patients, 18 years and above had a full-time or part-time job.

48.2%

More than three quarters of adults had either a high school diploma or college qualification / degree



78.7%

*Employment and education level excludes adults attending school.

At least least 300 biological children have been reported from 183 parents since the start of the registry. In 2018, 168 of these parents were alive and there were four (eight) children born respectively of a male (a female) parent with CF.

300

Kaplan E 1968, Barreto C 1991, Dreyfus DH 1996, Sawyer SM 2005,Janice E Whitty 2010, Jelin AC 2017

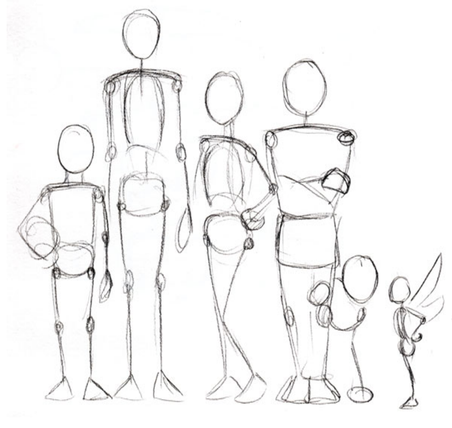
Due to advancements in CF care and novel treatments, including the CFTR treatments that are in the pipeline, people with CF continue to have better survival and life expectancy and with better quality of life.

Since inception of the registry, there have been about 1600 registered patients and 191 reported deaths, mostly from respiratory causes. About 10.5% of the deaths reported were in children below 18 years.

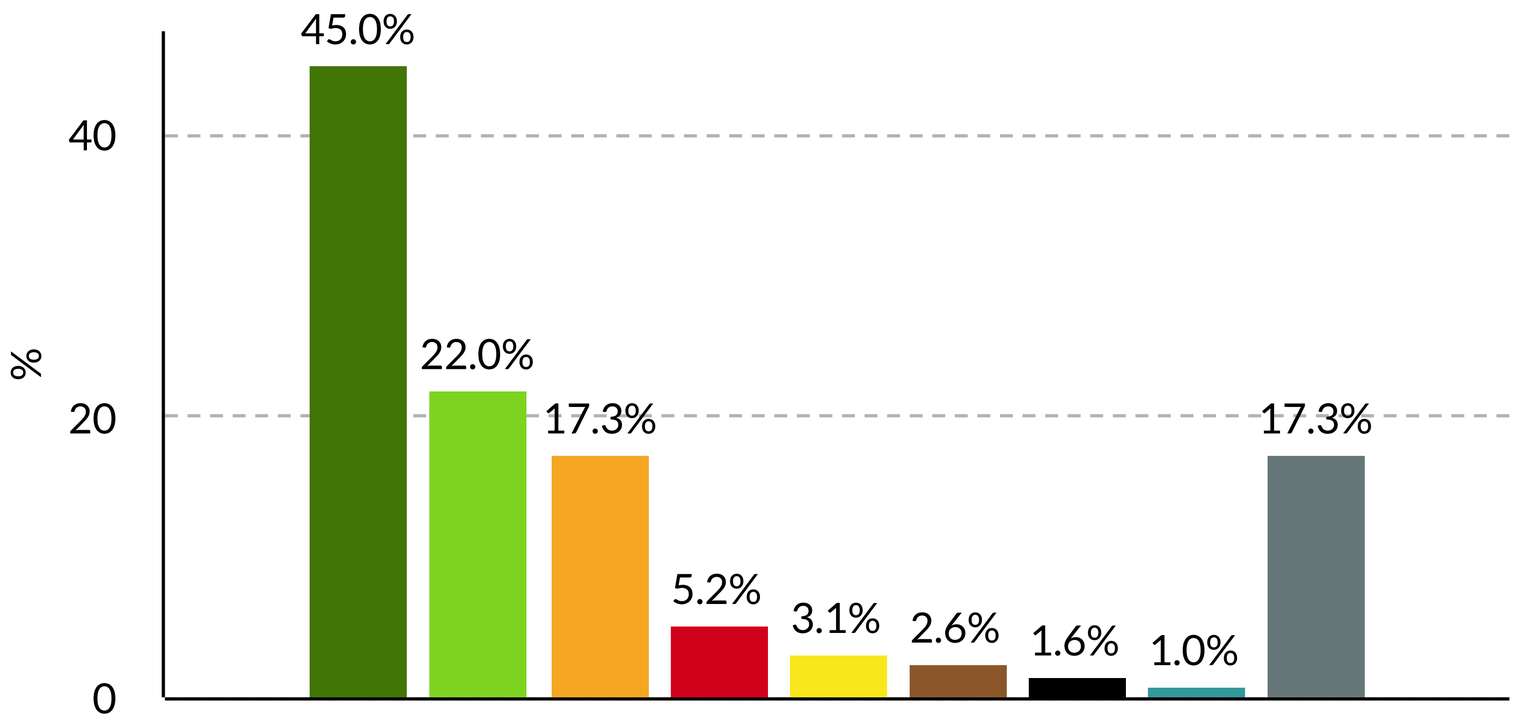
While survival estimates for patients with CF in Belgium are currently not available, the median predicted survival for children born with CF between 2014 and 2018 according to the CFF foundation is about 44.4 years.

This means that at least 50% of children born with CF from 2014 to 2018 will live to be 44.4 years or older.

Cystic Fibrosis Foundation 2018



Main causes of death*



*The causes of death are not mutually exclusive

Suggested reference: The Belgian Cystic Fibrosis Registry highlights 2018, Brussels, Belgium.

Source : Annual report Belgian Cystic Fibrosis Registry (BCFR) 2018.