Cystic Fibrosis Trust

UK Cystic Fibrosis Registry

Annual Data Report 2024 — Scotland

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UK Cystic Fibrosis Registry
Annual Data Report 2024 — Scotland

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Acknowledgements

First and foremost, UK Cystic Fibrosis Registry team would like to thank people with cystic fibrosis and their families for their support, as well as anyone who has generously donated to Cystic Fibrosis Trust. We would also like to express our gratitude to the UK cystic fibrosis centres and clinics for their continued dedication to obtaining consent and submitting data to the Registry.

Contact information

For more information about this report, or the UK Cystic Fibrosis Registry, please contact us: **registry@cysticfibrosis.org.uk @CFTrust**

Cystic Fibrosis Trust

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Executive summary

I am delighted to present the 2024 UK CF Registry Annual Data Report for Scotland.

The data represented in this report provides important insights into the health and clinical outcomes of people with CF cared for by CF teams in Scotland. The proportion of people with CF who had an annual review recorded on the Registry in 2024 was 76%, similar to that reported in 2023, but markedly lower than for the UK as a whole.

The UK CF Registry data continues to be a valuable resource for informing people with CF and their families, clinicians and policy makers, which is key to improving services.

The impact of improvements in CF therapies, with widespread use of CFTR modulator drugs, is reflected in the data reported. There is an increase in the proportion of patients in adult compared with paediatric services, as the CF population increases in size and grows older.

Highlights of the report include the following:

- The proportion of adults with CF in Scotland continues to increase with 66% of the Registry population over 16 years old
- There were 11 new diagnoses of CF in 2024 (section 1.1). Looking back over a longer period (2020-2024), there were 110 new diagnoses of CF, with more than half (65%) identified by newborn screening. In this larger cohort we get insight as to predominant initial presentations for those not diagnosed by newborn screening. Of the 39 people not identified by newborn screening, recurrent infection or bronchiectasis combined, were seen as the most common initial presentations.
- Lung health, represented by FEV1 has improved markedly between 2019 and 2024, as the number of people with CF eligible for and taking CF modulators has increased. This improvement is seen across the board, in nearly all ages. In 2024, almost 600 people with CF were taking CF modulator treatment in Scotland.
- Adults with CF have seen an increase in weight, particularly since the introduction of CF modulators. The data reported shows that more patients are achieving a normal or raised BMI in 2024 compared with 2019. As a consequence of improved nutrition, the use of supplementary feeding has decreased significantly in children and adults.
- Five women with CF, and partners of seven men with CF have had babies in 2024. Enabling people with CF to have informed opportunity to become parents is important.
- The numbers of people being evaluated and listed for lung transplant has decreased since the introduction of CF modulators, because of stabilisation of CF lung disease, following their introduction.

We are grateful to the people with CF in Scotland, for allowing their data to be collected, and to the clinical teams for recording the data. The data presented helps us all understand the changing landscape of CF care, to plan and provide the best care for people with CF in the future.

Dr Helen Rodgers
Consultant Respiratory Physician
Director Scottish Adult CF Service
Western General Hospital, Edinburgh

Introduction

This report is aimed at anyone who is interested in the health, care, and outcomes of people with cystic fibrosis (CF) receiving care at CF centres in Scotland. This includes people with CF, their families and clinical teams, healthcare managers, commissioners, and policy makers.

You can find a glossary of scientific and clinical terms on page 72.

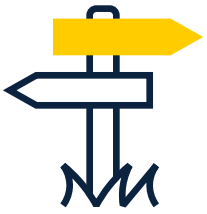
Cystic fibrosis

Cystic fibrosis is an inherited disease caused by a faulty version of a gene known as CFTR. The gene and the protein it makes help control the movement of salt and water in and out of cells. When the gene, and the protein it makes, is faulty, it can cause thicker mucus. One of the main areas affected is the lungs; over time this thick mucus blocks and damages airways, leading to infections and making it hard to breathe. People with CF may also develop other problems, such as liver disease or CF diabetes (CFD). Around 85% of people with CF also have difficulty digesting food.

UK Cystic Fibrosis Registry

The UK CF Registry has been sponsored and hosted by Cystic Fibrosis Trust since 2007. It is a database of consenting people with CF in the UK. The Registry collects demographic, treatment and health outcomes data. You can find a full list of the data items we collect at **cysticfibrosis.org.uk/registry**.

The purpose of the UK CF Registry is to improve the health of people with cystic fibrosis. This is done in a number of ways:



helping people with CF and their families understand CF, and make informed decisions

giving clinical teams the evidence they need to improve the quality of care

monitoring the safety and effectiveness of new treatments for cystic fibrosis

providing data for research to find out the best ways to treat cystic fibrosis

helping commissioners provide funding to NHS CF centres that is proportionate to the severity of their patients' condition

supporting clinical trials through feasibility studies and pragmatic data collection

Governance

The Registry Steering Committee (RSC) is responsible for making sure that the UK CF Registry is compliant with data protection legislation, and its Research Ethics Committee-approved Study Protocol. It also makes recommendations about the future development of the Registry.

The Registry Research Committee, which is a subcommittee of the RSC, assesses applications for data and guides the Registry research strategy.

Please see Appendix 2 of the UK Cystic Fibrosis Registry 2024 Annual Data Report.

Data are only recorded on the UK CF Registry if explicit written consent is given by the person with CF or, for a child, their parent or guardian.

When data are provided to third parties such as the NHS or university researchers, they are either anonymised (all identifiable data removed completely) or pseudonymised (all identifiable data replaced with a unique identification number). Pseudonymisation is used so that data can be traced back to what is in the 'live' database by the Registry team for the purposes of updating the data or answering queries. This means that the Registry data used for research, and the results cannot identify the people whose data are stored on the UK CF Registry.

If requests from pharmaceutical companies are granted, for research or submissions to regulators or the NHS, the data are analysed and aggregated by Registry statisticians and only summary data are provided.

Data collection

Data are entered onto the UK CF Registry by NHS employees at CF centres in the UK using a secure web portal.

Where can I find more information?

You can find out more about CF, and the UK CF Registry, at **cysticfibrosis.org.uk/registry**.

Section 1: Scotland-wide analysis

This section provides an overview of the cystic fibrosis (CF) population, health outcomes, and care in Scotland, with comparisons to the full CF population of the United Kingdom, including CF centres in England, Northern Ireland, Scotland, and Wales.

1.1 Summary of the UK Cystic Fibrosis Registry

	UK	Scotland
*	2024	2024
CF patients registered; n[1]	11381	969
Excluding diagnoses that year; n	11217	958
CF patients with an annual review; n(%)[2]	10424 (93)	731 (76)
New diagnoses		
All newly diagnosed patients; n	164	11
Patients identified by NBS; n	123	6
Patients diagnosed age 16 years or older; n[3]	17	<5
Demographics		
Age in years; median (IQR)[4]	23 (12, 35)	22 (13, 35)
Adults aged 16 years and over; %[5]	65	66
Males; %[5]	53	54
Genotyped; %[5]	99	100
Total deaths during annual review year; n(%)	51 (0.4)	<5
Age at death in years; median (IQR)	42 (31, 51)	**

Annual review: A Registry annual review form contains a combination of data relating to a person with CF's yearly annual review appointment at their CF centre, and their clinical care and health over the past 12 months.

Notes:

* Unless otherwise stated in a footnote, all proportions are calculated from all CF patients registered (row 1).

** Not provided due to very low sample size.

1 Number of patients alive on 1 January in the given year diagnosed with CF, with at least 1 annual review in either the given year or the previous 2 annual review years or at least 1 recorded encounter in the given year, plus any newly diagnosed and/or newly consented individuals. We use this definition as a proxy for the sub-population of people with CF who are actively engaged with NHS services.

2 Newly diagnosed patients in the given year may not have their first annual review in the same year, so the proportion with an annual review is calculated from the total registered excluding those diagnosed in the given year.

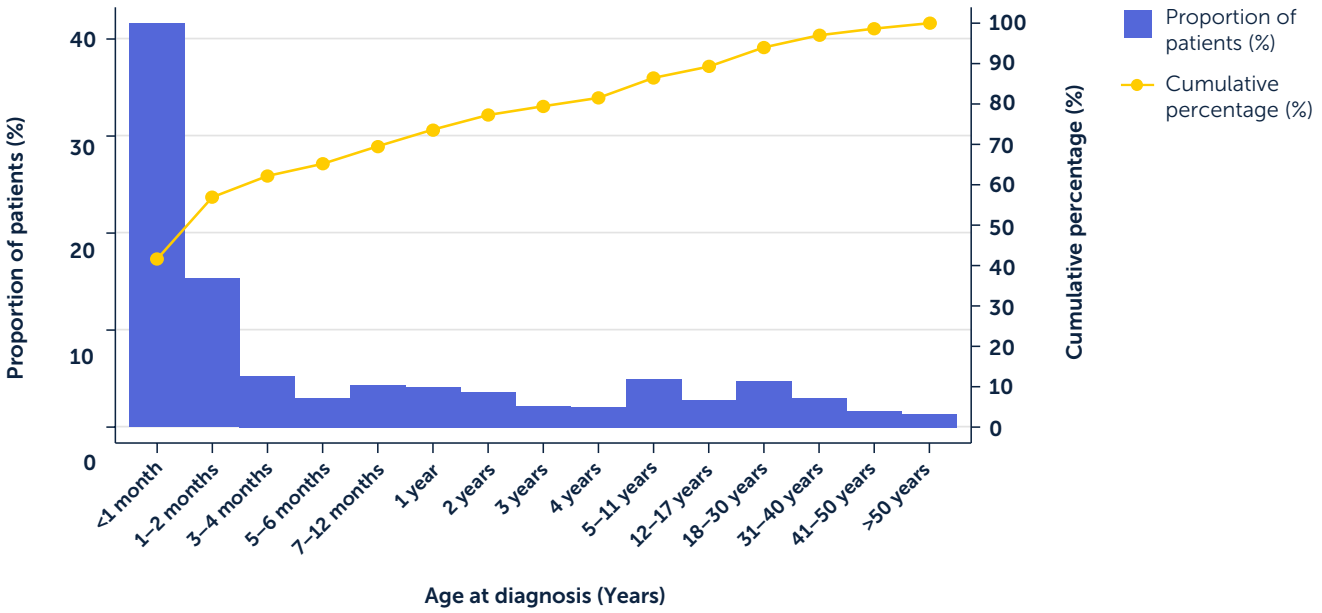
3 This figure is the total number of individuals newly diagnosed in the given year who were 16 years or older at the time of diagnosis.

4 Age is taken at the given year's annual review; if the individual did not have an annual review during the year in question, their age was taken on 1 January of the given year.

5 Calculated from all registered patients in the given year. Figures may be different from previous reports as they were previously calculated from patients with an annual review in the given year.

Diagnosis of cystic fibrosis

1.2 Age at diagnosis
N=968



1.3 Mode of presentation
N=110

The following table shows the most frequent modes of presentation for people diagnosed between 2020 – 2024. Individuals included in this table were registered and diagnosed between 2020 – 2024. Patients not diagnosed through newborn screening (NBS) may present multiple symptoms so the categories are not mutually exclusive, and percentages may not add up to 100.

	Age <1 year at diagnosis	Age ≥1 year & < 16 years at diagnosis	Age ≥16 years at diagnosis
Total patients	**78**	**5**	**27**
Number diagnosed by newborn screening (NBS)[1]	68	<5	0
Total non-NBS	10	<5	27

Individuals not diagnosed by NBS[2]	**10**	**<5**	**27**
Bronchiectasis	0	<5	8 (29.6)
Persistant or acute respiratory infection	<5	<5	<5
Genotype	0	0	5 (18.5)
Meconium ileus	5 (50.0)	0	0
Family history	<5	0	<5
Pancreatitis	0	0	<5

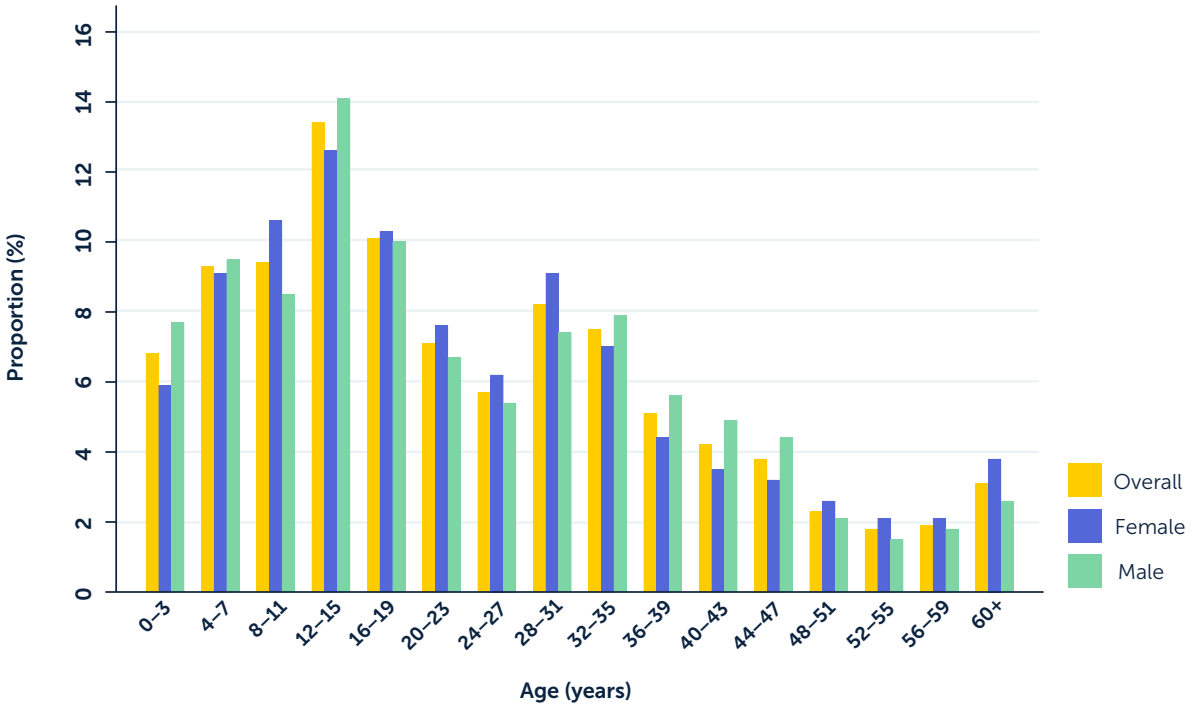
1 If the method of diagnosis was genotyping and the age of diagnosis was <1 year, this was counted as a diagnosis by newborn screening (n=19)

2 all other modes of presentation were reported with frequency fewer than five : Unknown, Prenatal, Abnormal stools / fatty stool (steatorrhea)/malabsotption, Fertility and Failure to thrive.

Demographics

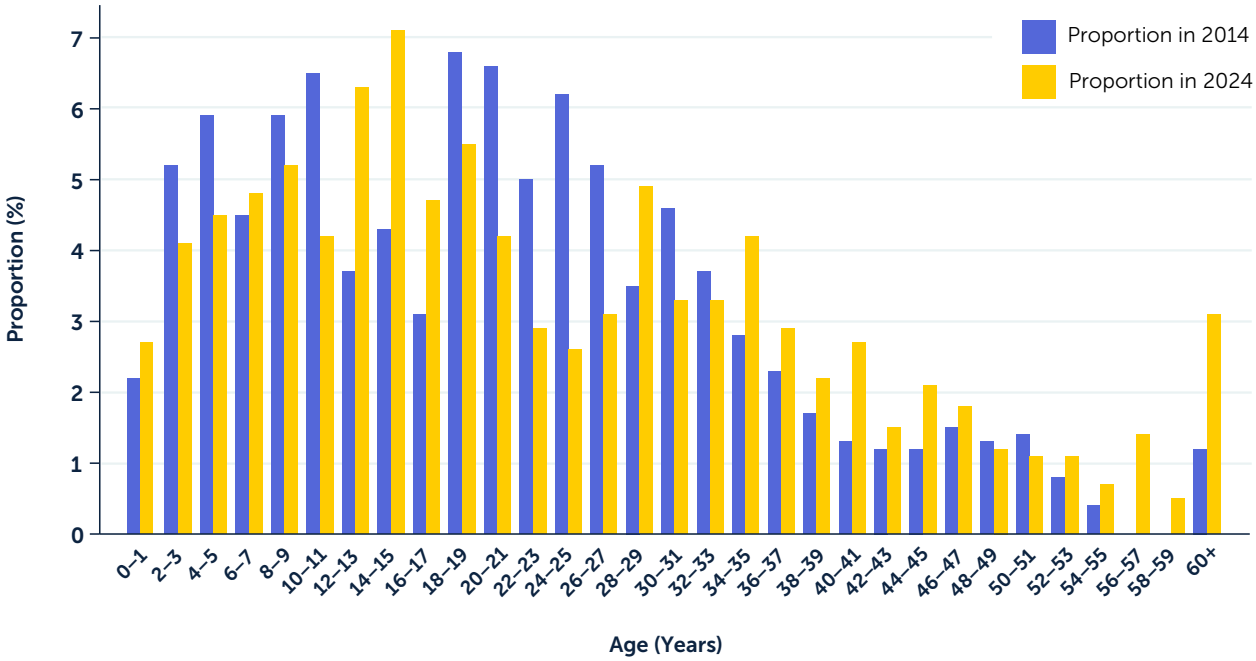
1.4 Age distribution by sex
N=731

This graph shows the age distribution by sex for people with an annual review in 2024.



Age	All; n (%)	Females; n (%)	Males; n (%)
0-3	50 (6.8)	20 (5.9)	30 (7.7)
4-7	68 (9.3)	31 (9.1)	37 (9.5)
8-11	69 (9.4)	36 (10.6)	33 (8.5)
12-15	98 (13.4)	43 (12.6)	55 (14.1)
16-19	74 (10.1)	35 (10.3)	39 (10.0)
20-23	52 (7.1)	26 (7.6)	26 (6.7)
24-27	42 (5.7)	21 (6.2)	21 (5.4)
28-31	60 (8.2)	31 (9.1)	29 (7.4)
32-35	55 (7.5)	24 (7.0)	31 (7.9)
36-39	37 (5.1)	15 (4.4)	22 (5.6)
40-43	31 (4.2)	12 (3.5)	19 (4.9)
44-47	28 (3.8)	11 (3.2)	17 (4.4)
48-51	17 (2.3)	9 (2.6)	8 (2.1)
52-55	13 (1.8)	7 (2.1)	6 (1.5)
56-59	14 (1.9)	7 (2.1)	7 (1.8)
60+	23 (3.1)	13 (3.8)	10 (2.6)
<16	285 (39.0)	130 (38.1)	155 (39.7)
≥16	446 (61.0)	211 (61.9)	235 (60.3)
<18	319 (43.6)	149 (43.7)	170 (43.6)
≥18	412 (56.4)	192 (56.3)	220 (56.4)
Overall	**731**	**341**	**390**

1.5 Age distribution of the UK CF population in 2014 and 2024
N=731 in 2024, N=782 in 2014



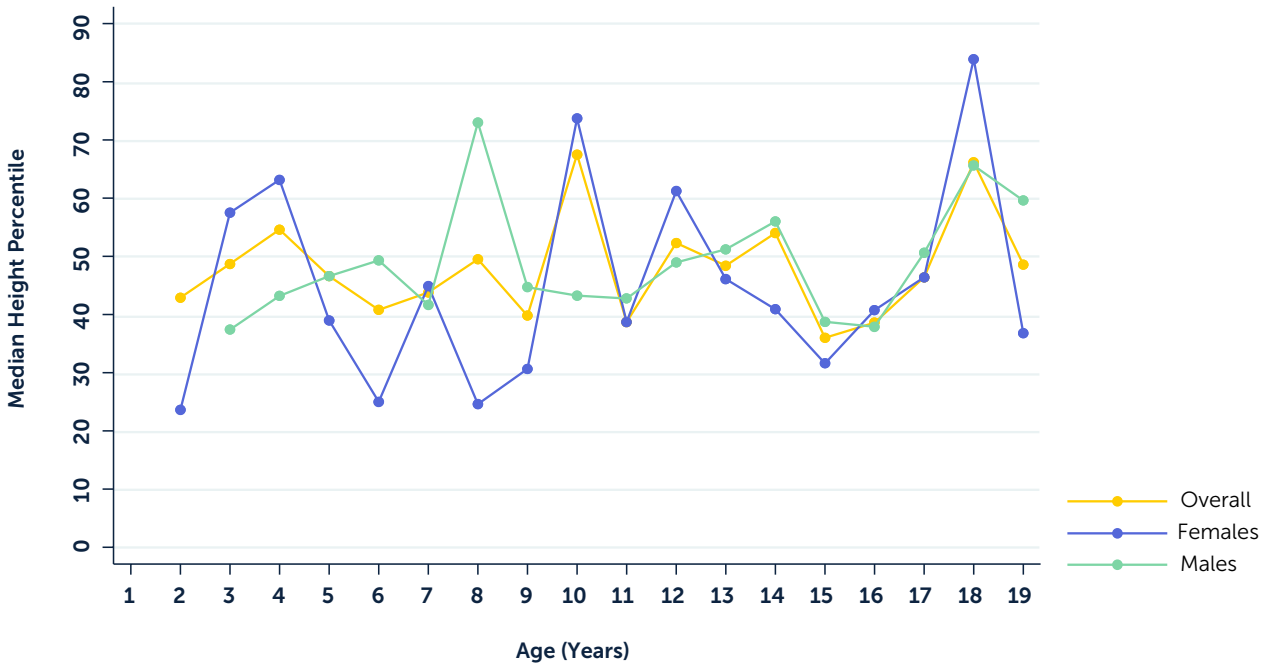
1.6 Ethnicity

Ethnicity n (%)	2014	2019	2024
Total	782	868	731
Total known[1]	781	859	724
White	762 (97.6)	842 (98.0)	706 (97.5)
Asian	12 (1.5)	11 (1.3)	13 (1.8)
Black	0	0	0
Mixed[2]	<5	<5	5 (0.7)
Other	-*	<5	0

* Redacted to adhere to statistical disclosure guidelines
1 Proportions are calculated from total known ethnicities
2 Further detail on mixed ethnicity categories were collected from 2016 onwards

1.7 Height percentiles of children and young people (age 2-19 years)[1] N=339

The following chart and table show the height percentiles of people with CF, aged 19 and under, in relation to UK growth data for the general population. If a person with CF is on the 40th percentile, only 40% of people the same age are their height or shorter; 60% are taller.



Age	Overall	Median	IQR	Female	Median	IQR	Male	Median	IQR
2	9	42.9	23.6-46.0	-*	23.6	21.1-44.8	<5	-*	-*
3	17	48.7	31.8-65.7	6	57.5	45.2-69.0	11	37.4	10.0-65.7
4	14	54.6	39.9-76.1	8	63.2	54.6-91.7	6	43.2	38.1-47.2
5	19	46.6	16.2-80.0	8	39.0	10.1-83.4	11	46.6	17.6-71.7
6	13	40.8	22.6-70.0	5	25.0	22.6-51.0	8	49.3	29.1-74.4
7	21	43.8	21.3-68.6	9	44.9	24.0-53.3	12	41.7	16.9-84.4
8	19	49.5	22.1-78.7	9	24.6	17.8-44.2	10	73.0	49.5-87.3
9	16	39.8	20.5-50.7	8	30.7	14.8-45.2	8	44.7	28.2-66.8
10	12	67.5	17.3-75.5	6	73.7	17.9-93.2	6	43.2	3.0-72.3
11	19	38.7	16.1-76.5	11	38.7	16.1-84.1	8	42.8	22.3-56.7
12	23	52.3	25.6-80.3	9	61.2	27.0-67.5	14	49.0	25.6-80.3
13	22	48.4	26.4-62.6	11	46.1	26.4-52.9	11	51.2	21.5-81.5
14	23	54.0	27.3-70.0	10	40.9	21.7-88.6	13	56.0	39.3-65.8
15	27	36.0	15.8-52.8	11	31.6	13.3-48.8	16	38.8	20.2-59.3
16	15	38.6	31.0-59.3	10	40.8	31.0-59.9	5	37.9	34.9-39.8
17	19	46.4	22.5-61.3	9	46.4	22.5-59.7	10	50.6	34.6-61.3
18	20	66.1	34.2-85.5	7	83.9	59.4-85.5	13	65.6	25.6-74.1
19	20	48.6	22.5-62.6	9	36.8	18.0-49.1	11	59.6	26.9-86.5
Overall	**328****	**46.5**	**21.6-70.0**	**151**	**44.9**	**20.6-69.0**	**177**	**49.5**	**23.3-70.0**

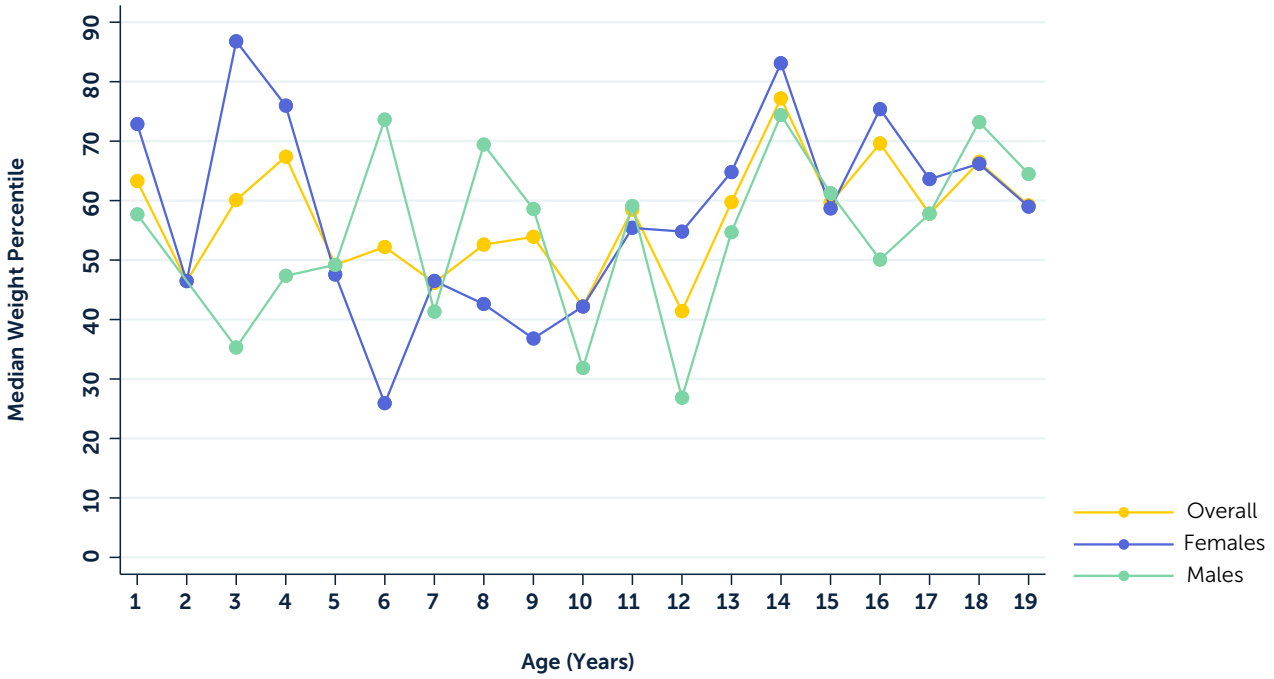
* Redacted to adhere to statistical disclosure guidelines and / or summary statistics not shown for frequencies less than 5.

** Number with non-missing data and age 2 to 19 years.

1 Based on UK-WHO growth charts, 1990 (updated 1996).

1.8 Weight percentiles of children and young people (age 1-19 years)[1] N=356

The following chart and table show the weight percentiles of people with CF, aged 19 and under, in relation to the UK growth data for the general population. If a person with CF is on the 40th percentile, only 40% of people the same age are their weight or lower; 60% weigh more.



	Overall			Female			Male		
Age	n	Median	IQR	n	Median	IQR	n	Median	IQR
1	15	63.3	14.7-82.5	5	72.9	47.3-89.8	10	57.7	14.7-71.7
2	10	46.4	28.2-83.5	6	46.4	28.2-83.5	<5	-*	-*
3	17	60.1	34.4-80.7	6	86.8	60.1-97.0	11	35.3	31.0-79.6
4	14	67.4	47.8-78.6	8	75.9	62.8-83.9	6	47.3	34.6-71.6
5	19	49.2	34.5-74.2	8	47.6	31.2-64.9	11	49.2	39.1-80.5
6	13	52.2	24.8-82.2	5	25.9	24.8-34.0	8	73.6	31.5-85.7
7	21	46.2	17.3-65.4	9	46.5	26.9-65.4	12	41.3	8.4-72.2
8	19	52.6	34.1-89.8	9	42.6	24.0-94.6	10	69.4	50.7-89.1
9	16	53.9	20.0-79.7	8	36.8	17.1-70.7	8	58.6	35.8-84.9
10	12	42.2	10.6-58.8	6	42.2	18.2-58.1	6	31.9	9.7-59.6
11	19	58.5	31.8-78.1	11	55.4	30.3-83.7	8	59.1	42.8-62.3
12	23	41.4	17.0-86.6	9	54.8	28.3-75.2	14	26.9	17.0-90.4
13	22	59.8	29.8-82.7	11	64.8	25.4-88.7	11	54.7	41.8-82.7
14	23	77.2	42.2-93.1	10	83.1	42.2-96.1	13	74.4	51.3-91.1
15	27	59.6	33.7-78.0	11	58.7	34.8-77.6	16	61.2	29.0-81.8
16	15	69.6	37.7-83.0	10	75.4	42.1-88.3	5	50.1	25.1-69.6
17	17	57.8	52.4-81.2	8	63.6	53.9-83.5	9	57.8	49.1-75.7
18	19	66.5	41.1-84.7	7	66.2	41.1-75.9	12	73.2	39.7-87.0
19	18	59.2	27.7-80.7	7	59.0	45.0-69.6	11	64.5	19.9-98.0
Overall	**339****	**58.1**	**31.0-80.9**	**154**	**58.4**	**33.5-81.2**	**185**	**57.8**	**27.7-80.7**

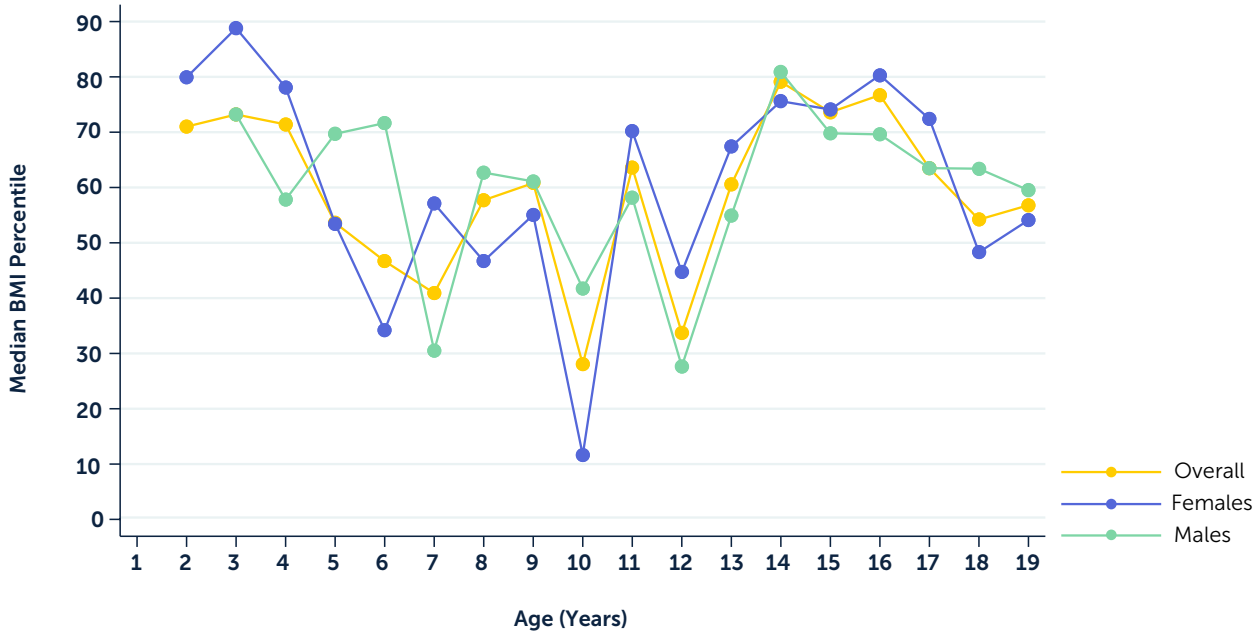
* Redacted to adhere to statistical disclosure guidelines and / or summary statistics not shown for frequencies less than 5.

** Number with non-missing data and age 1 to 19 years.

1 Based on UK-WHO growth charts, 1990 (updated 1996).

1.9a Body Mass Index (BMI) percentiles in children and young people (age 2-19 years)[1]
N=339

The following chart and table show the BMI percentiles of people with CF, aged 19 and under, in relation to the UK growth data for the general population. If a person with CF is on the 40th percentile, it means that only 40% of the population at the same age are their BMI or lower; so 60% have a higher BMI.



	Overall			Female			Male		
Age	n	Median	IQR	n	Median	IQR	n	Median	IQR
2	9	71.0	58.7-87.9	5	79.9	71.0-90.4	<5	-*	-*
3	17	73.2	52.1-87.4	6	88.8	61.6-97.3	11	73.2	50.7-81.7
4	14	71.4	48.3-87.5	8	78.1	49.2-89.1	-*	57.8	48.3-87.5
5	19	53.6	49.8-78.9	8	53.4	46.8-75.1	11	69.7	49.8-80.8
6	13	46.7	34.2-81.6	5	34.2	16.7-46.7	8	71.7	43.2-92.2
7	21	40.9	20.8-68.2	9	57.1	35.7-67.0	12	30.5	17.5-72.8
8	19	57.7	32.4-84.2	9	46.7	42.3-95.5	10	62.7	32.4-82.5
9	16	60.8	40.7-89.1	8	55.1	26.8-82.6	8	61.1	46.5-90.4
10	12	28.0	7.5-48.5	6	11.7	2.5-23.5	6	41.8	32.6-49.3
11	19	63.6	39.4-76.6	11	70.2	39.4-74.4	8	58.2	46.8-84.1
12	23	33.7	16.9-88.6	9	44.7	27.8-71.2	14	27.6	16.9-91.7
13	22	60.6	35.5-86.6	11	67.4	23.7-88.9	11	54.9	35.5-86.6
14	23	79.1	49.0-95.2	10	75.6	60.8-93.0	13	80.9	49.0-95.5
15	27	73.6	40.5-88.7	11	74.1	61.8-88.7	16	69.8	40.1-88.1
16	15	76.7	54.2-89.9	10	80.3	63.5-90.1	5	69.6	30.3-81.2
17	17	63.5	52.2-87.5	8	72.4	57.9-95.5	9	63.5	45.4-85.3
18	19	54.2	40.4-78.0	7	48.3	21.0-67.7	12	63.4	40.9-93.6
19	18	56.8	36.5-92.0	7	54.1	50.4-92.0	11	59.5	9.4-93.9
Overall	**323****	**61.6**	**35.8-85.3**	**148**	**63.6**	**41.6-86.1**	**175**	**57.7**	**32.4-85.3**

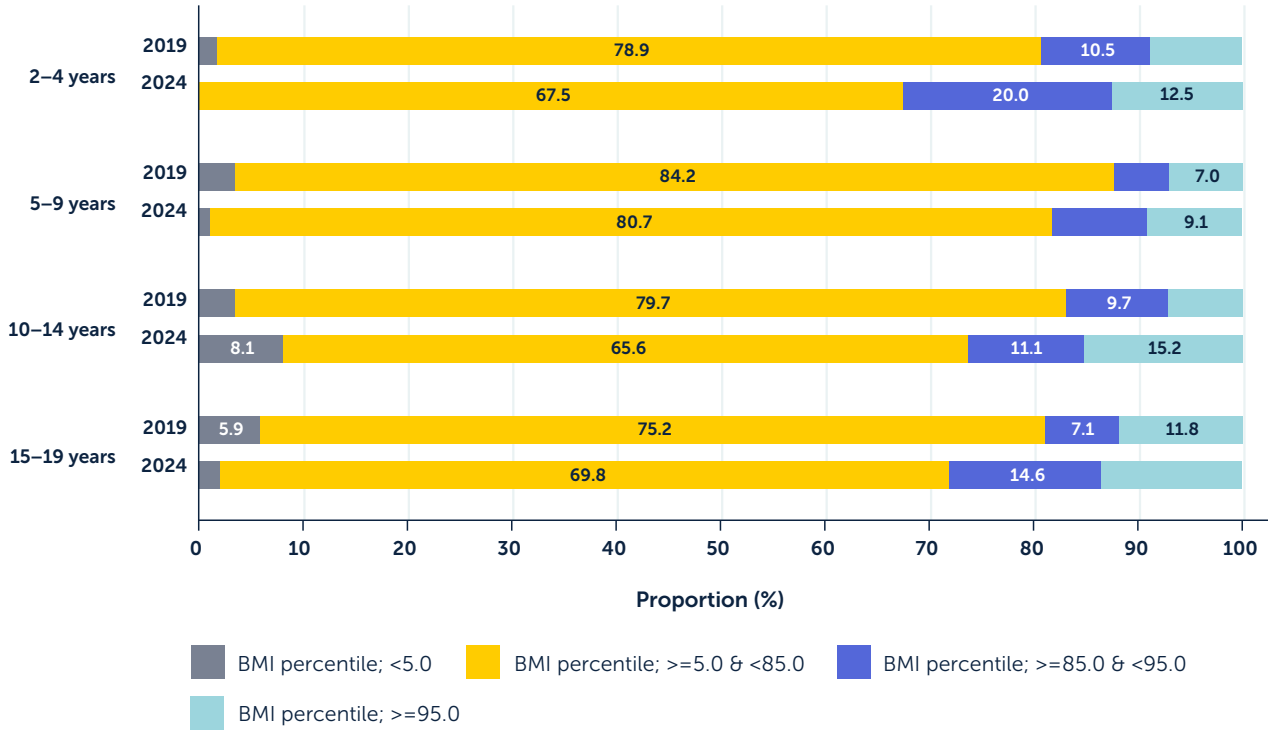
* Redacted to adhere to statistical disclosure guidelines and / or summary statistics not shown for frequencies less than 5.

** Number with non-missing data and age 2 to 19 years.

1 Based on UK-WHO growth charts, 1990 (updated 1996).

1.9b Body Mass Index (BMI) percentiles in children and young people (age 2-19 years)[1] for 2019 and 2024

The following graph shows the change in BMI groups for children and young people with CF in 2019 and 2024.



			BMI category by age and year : n(%)			
Age group	Year	Total number of people in each age group**	BMI percentile; <5.0	BMI percentile; >=5.0 & <85.0	BMI percentile; >=85.0 & <95.0	BMI percentile; >=95.0
2-4 years	2019	57	<5	45 (78.9)	6 (10.5)	-*
	2024	40	0	27 (67.5)	8 (20.0)	5 (12.5)
5-9 years	2019	114	<5	96 (84.2)	-*	8 (7.0)
	2024	88	<5	71 (80.7)	-*	8 (9.1)
10-14 years	2019	113	<5	90 (79.7)	11 (9.7)	-*
	2024	99	8 (8.1)	65 (65.7)	11 (11.1)	15 (15.0)
15-19 years	2019	85	5 (5.9)	64 (75.3)	6 (7.1)	10 (11.8)
	2024	96	<5	67 (70.0)	14 (15.0)	-*

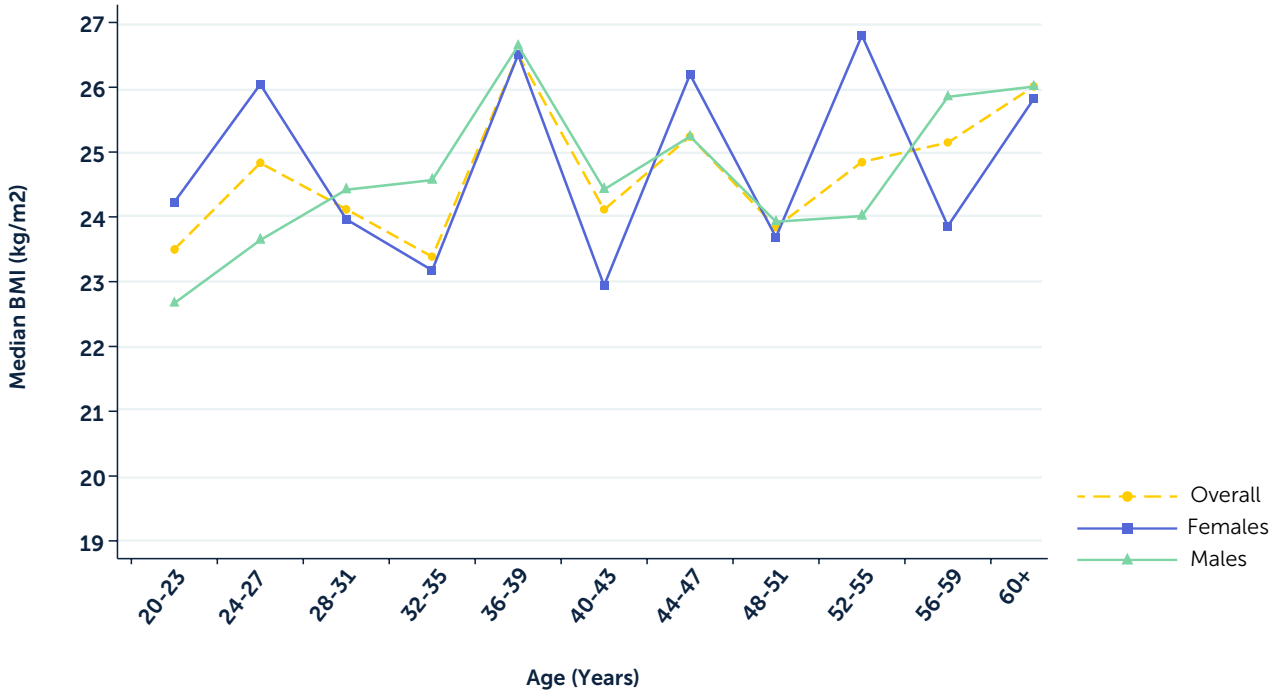
* Redacted to adhere to statistical disclosure guidelines.

** Number with non-missing data and age 2 to 19 years.

1 Based on UK-WHO growth charts, 1990 (updated 1996).

1.10a Body Mass Index (BMI) in adults ages 20 and older N=372

The following chart and table show the BMI of people with CF aged 20 and over.

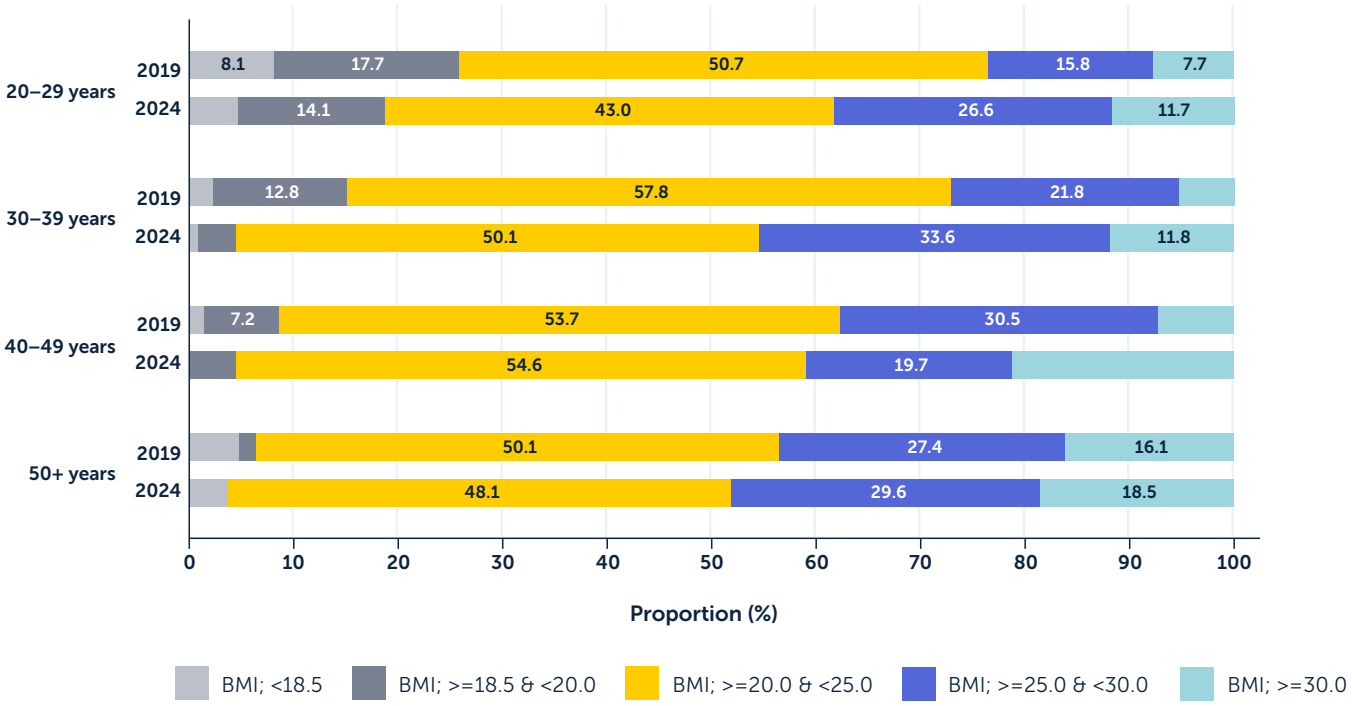


	Overall			Female			Male		
Age	n	Median	IQR	n	Median	IQR	n	Median	IQR
20-23	51	23.5	20.9-25.2	25	24.2	20.9-25.4	26	22.7	20.9-24.2
24-27	42	24.8	20.8-28.8	21	26.0	21.5-29.1	21	23.6	20.7-27.8
28-31	58	24.1	22.0-26.9	31	24.0	21.5-27.6	27	24.4	22.3-26.3
32-35	52	23.4	22.0-25.4	23	23.2	20.9-24.0	29	24.6	22.4-25.7
36-39	35	26.5	23.5-29.4	13	26.5	22.8-27.8	22	26.6	23.9-29.4
40-43	30	24.1	22.3-26.1	11	22.9	22.3-25.2	19	24.4	21.5-29.0
44-47	27	25.2	22.7-30.5	10	26.2	21.5-32.2	17	25.2	22.8-26.9
48-51	16	23.8	23.1-27.1	9	23.7	23.2-25.4	7	23.9	22.0-31.9
52-55	13	24.8	23.3-27.7	7	26.8	23.4-29.5	6	24.0	23.2-25.8
56-59	12	25.1	23.4-29.0	5	23.9	23.4-25.3	7	25.9	23.4-29.4
60+	22	26.0	23.8-29.6	12	25.8	22.7-32.8	10	26.0	24.5-29.5
Overall	**358***	**24.2**	**22.0-27.6**	**167**	**23.9**	**21.7-27.4**	**191**	**24.6**	**22.3-27.8**

* Number with non-missing data.

1.10b Body Mass Index (BMI) in adults for 2019 and 2024

The following graph shows the change in the proportion of people in each BMI group in 2019 and 2024.



			BMI category by age and year : n(%)				
Age group	Year	Total number of people in each age group**	BMI; <18.5	BMI; >=18.5 & <20.0	BMI; >=20.0 & <25.0	BMI; >=25.0 & <30.0	BMI; >=30
20-29 years	2019	209	17 (8.1)	37 (17.7)	106 (50.7)	33 (15.8)	16 (7.7)
	2024	128	6 (4.7)	18 (14.1)	55 (43.0)	34 (26.6)	15 (11.7)
30-39 years	2019	133	<5	17 (12.8)	77 (57.9)	29 (21.8)	-*
	2024	110	<5	<5	55 (50.0)	37 (33.6)	13 (11.8)
40-49 years	2019	69	<5	5 (7.2)	37 (53.6)	21 (30.4)	-*
	2024	66	0	<5	36 (54.5)	13 (19.7)	-*
50+ years	2019	62	<5	<5	31 (50.0)	17 (27.4)	10 (16.1)
	2024	54	<5	0	26 (48.1)	16 (29.6)	10 (18.5)

* redacted to adhere to statistical disclosure guideline.

** Number with non-missing data.

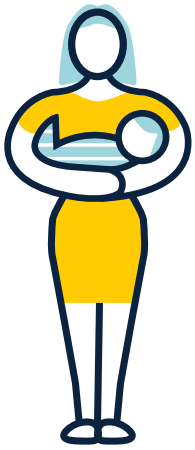
1.11 Education and employment in adults (≥16 years) N=446

The following table shows how people with CF aged 16 and older reported their education and employment status in 2024.

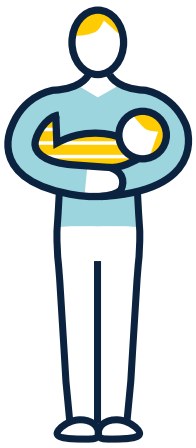
	Overall	Male	Female
Numbers of patients; n	446	235	211
Number who completed questionnaire; n(%)[1]	446 (100.0)	235 (100.0)	211 (100.0)
Full-time employment; n(%)	192 (43.0)	124 (52.8)	68 (32.2)
Part-time employment; n(%)	75 (16.8)	24 (10.2)	51 (24.2)
Student; n(%)	82 (18.4)	37 (15.7)	45 (21.3)
Homemaker; n(%)	9 (2.0)	<5	9 (4.3)
Unemployed; n(%)	59 (13.2)	32 (13.6)	27 (12.8)
Disabled; n(%)	<5	<5	<5
Retired; n(%)	16 (3.6)	10 (4.3)	6 (2.8)
Volunteer; n(%)	<5	<5	<5
Unknown entered; n(%)	10 (2.2)	6 (2.6)	<5
No. in work or study; n(%)	**349 (78.3)**	**185 (78.7)**	**164 (77.7)**

1.12 Parenthood

	2021	2022	2023	2024
Women with CF who had babies; n	7	8	5	5
Men with CF who became fathers; n	<5	<5	<5	7



Five women with cystic fibrosis had babies in Scotland during 2024



Seven men with cystic fibrosis became fathers in Scotland during 2024

1 All proportions below are calculated from the total who completed the questionnaire

Lung health

For people with CF, thickened mucus in the lungs is linked to repeat or chronic infections. This can cause permanent damage, making it harder to breathe.

In CF, the condition of the lungs is often measured using FEV_1; the Forced Expiratory Volume of air in the first second of a forced exhaled breath. In this report, an FEV_1% predicted is based on the FEV_1 we would expect for a person without CF of the same age, gender, height, and ethnicity.

A person with CF who has FEV_1% predicted of 100% can breathe out the same amount of air in the first second of an exhaled breath as we would expect from a comparable person without cystic fibrosis. A person with FEV_1% predicted of 50% breathes out half the volume of air as a comparable person without cystic fibrosis.

For people with CF, an FEV_1% predicted of 85% or higher is the target, as this indicates normal or near-normal lung health. Each individual with CF will have their own FEV_1 target, based on their own lung function results and trends.

An aim of CF care is to prevent FEV_1% predicted from falling as much as possible, for as long as possible. This is often a team effort between people with CF, their family, and their medical team, which can include doctors, nurses, physiotherapists, dietitians, and psychologists.

The FEV_1% predicted values shown in this report are calculated using an equation called Global Lungs Initiative, or 'GLI'[1].

1 Quanjer et al. Eur respir J. 2012 40(6):1324-1343

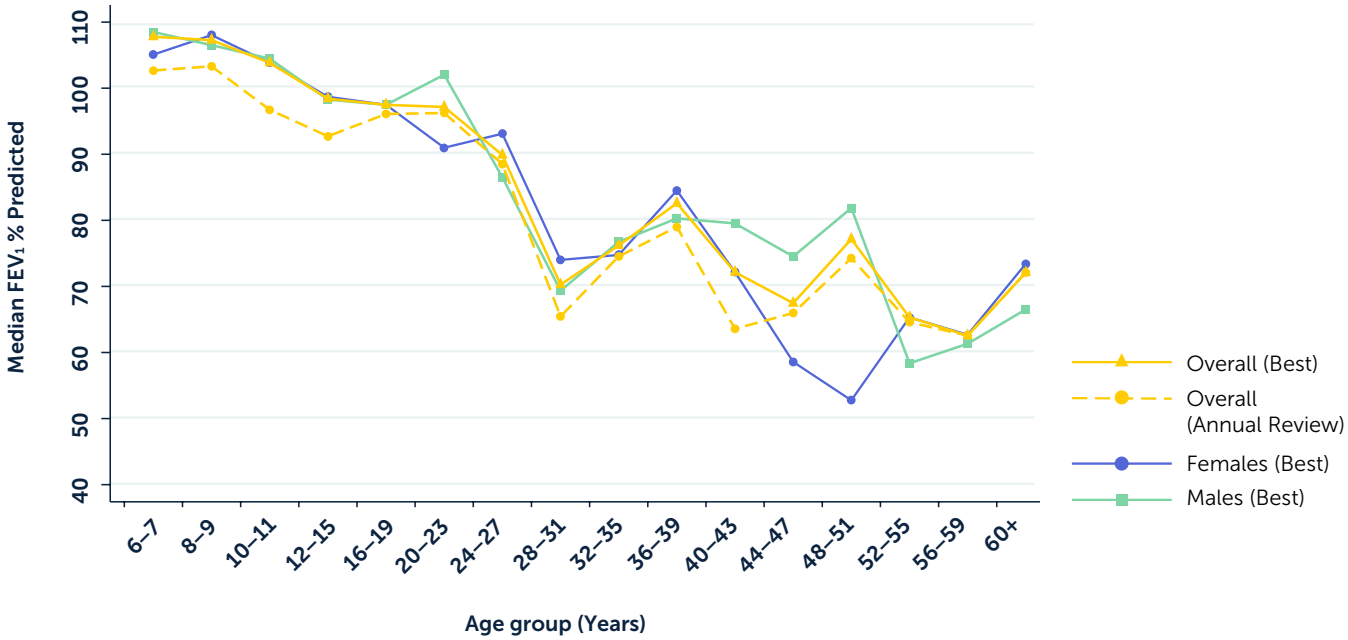
1.13 FEV_1% predicted (GLI equations) at annual review in patients aged 6 years and older who have not had a lung transplant
N=629

People with CF who have had lung transplants are excluded, as their new 'non-CF' lungs may have lung health similar to a person without cystic fibrosis.

	Overall			Female			Male		
Age (years)	n	Median	IQR	n	Median	IQR	n	Median	IQR
6-7	33	102.7	89.2-113.3	13	100.3	96.3-107.8	20	106.7	89.1-115.9
8-9	34	103.3	96.7-110.7	17	101.6	93.1-106.5	17	106.5	101.1-115.4
10-11	29	96.7	92.4-105.3	16	96.5	92.1-103.8	13	97.5	93.5-111.1
12-15	91	92.7	86.0-101.9	39	94.5	87.9-101.4	52	92.2	83.5-102.8
16-19	73	96.1	86.1-102.2	34	94.2	85.3-101.0	39	96.1	86.1-103.4
20-23	51	96.2	70.6-103.7	26	88.3	55.5-99.6	25	101.2	82.9-105.0
24-27	41	88.5	62.6-97.5	20	91.2	61.0-96.8	21	80.6	70.8-100.7
28-31	59	65.4	42.3-84.1	31	65.4	36.1-91.4	28	64.5	45.8-79.1
32-35	52	74.5	50.1-89.4	22	74.5	63.2-87.2	30	73.7	44.8-90.6
36-39	35	79.0	68.9-87.8	14	80.7	70.5-87.8	21	77.8	61.5-86.8
40-43	27	63.6	50.5-89.6	11	57.9	46.3-84.6	16	67.4	53.5-94.5
44-47	22	66.0	52.5-93.4	7	58.6	48.1-91.8	15	66.2	56.3-100.1
48-51	14	74.3	51.0-75.6	8	52.2	39.1-75.1	6	75.6	73.8-77.6
52-55	12	64.6	47.2-73.6	6	64.6	48.7-68.3	6	58.4	37.3-78.8
56-59	13	62.5	45.5-68.1	7	62.7	45.5-79.4	6	61.3	34.2-62.6
60-63	14	72.2	60.9-81.3	9	76.5	61.5-81.3	5	62.1	50.5-70.9
64-67	190	97.4	88.7-106.5	86	96.6	89.5-104.7	104	98.0	88.1-108.9
68+	424	79.1	56.8-96.1	198	79.3	55.6-94.4	226	78.9	57.0-97.0
<16	220	97.2	88.6-105.8	103	96.5	89.0-104.7	117	97.5	88.2-108.1
≥16	380	77.3	55.5-94.3	177	76.6	53.5-93.5	203	78.1	56.4-96.1
Overall	**600***	**88.0**	**68.1-100.3**	**280**	**87.9**	**65.8-98.5**	**320**	**88.0**	**70.7-102.0**

* Number with non-missing data.

1.14 Best FEV_1% predicted (GLI equations) in patients aged 6 years and older who have not had a lung transplant
N=629

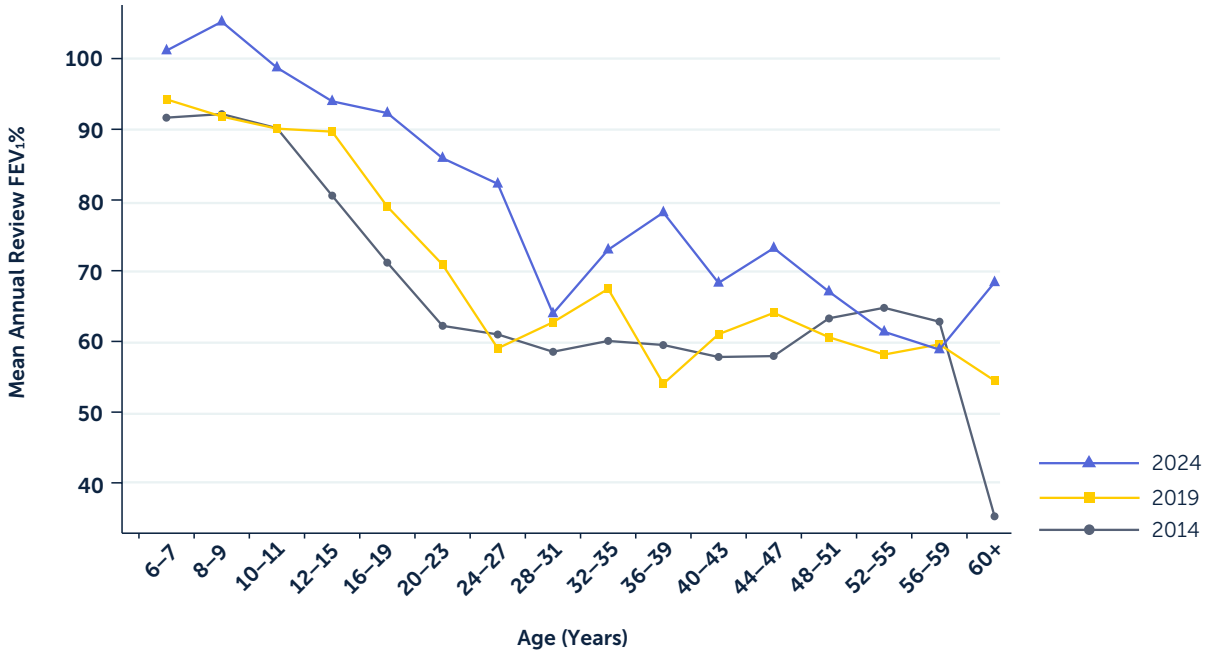


	Overall			Female			Male		
Age (years)	n	Median	IQR	n	Median	IQR	n	Median	IQR
6-7	35	107.8	94.8-119.5	15	105.1	98.5-120.1	20	108.5	92.6-118.4
8-9	36	107.3	101.2-115.1	17	108.0	98.5-112.7	19	106.5	101.3-116.2
10-11	31	103.8	95.7-107.7	17	103.8	95.0-106.4	14	104.5	96.3-114.6
12-15	95	98.4	89.9-106.8	41	98.7	90.3-106.9	54	98.3	89.6-106.6
16-19	73	97.5	88.4-104.0	34	97.5	87.7-107.1	39	97.5	88.4-103.8
20-23	52	97.2	77.5-106.4	26	91.0	65.5-101.9	26	102.0	88.4-107.4
24-27	41	89.9	63.0-98.5	20	93.1	62.8-98.2	21	86.5	70.8-106.3
28-31	59	70.3	49.0-85.9	31	74.0	49.0-95.0	28	69.3	50.8-81.2
32-35	53	76.2	53.4-91.2	23	74.8	64.2-89.9	30	76.8	47.2-91.2
36-39	35	82.6	71.7-93.7	14	84.5	74.4-91.4	21	80.3	67.5-93.7
40-43	27	72.2	55.6-95.7	11	72.2	48.7-84.6	16	79.5	59.5-98.1
44-47	23	67.5	54.1-94.7	7	58.6	48.1-94.1	16	74.5	57.2-98.9
48-51	16	77.2	50.3-85.8	8	52.8	43.2-85.6	8	81.9	74.2-85.8
52-55	12	65.3	47.2-74.4	6	65.3	48.7-68.3	6	58.4	40.9-80.5
56-59	13	62.5	45.9-72.4	7	62.7	45.7-84.1	6	61.3	45.9-72.4
60+	21	72.1	59.9-81.3	11	73.4	60.9-81.3	10	66.5	53.2-84.6
<16	197	102.6	92.9-109.7	90	103.4	92.9-108.5	107	102.2	92.5-111.3
≥16	425	83.0	62.1-97.8	198	83.1	63.0-97.0	227	83.0	61.2-98.6
<18	230	102.1	92.1-108.9	108	102.1	91.9-108.1	122	102.0	92.5-111.1
≥18	192	88.8	65.3-101.2	93	89.0	65.1-99.8	99	88.5	68.5-102.1
Overall	**622***	**91.0**	**72.5-103.8**	**288**	**90.6**	**72.0-102.7**	**334**	**91.1**	**73.5-104.1**

* Number with non-missing data.

1.15 FEV_1% predicted (GLI equations) over time in patients aged 6 years and older who have not had a lung transplant
N=629 in 2024, N=737 in 2019, N=635 in 2014*

As we learn more about CF and how to treat it, we hope to improve the outcomes of people with the condition. The chart below shows how FEV_1 in 2024 compares to Registry data from 2014 and 2019.



	2014		2019		2024		
Age (years)	n	FEV_1% : Mean (SD)	n	FEV_1% : Mean (SD)	n	FEV_1% : Mean (SD)	p-values (t-test**)
6-7	36	91.6 (16.1)	37	94.2 (16.1)	33	101.1 (17.9)	0.094
8-9	42	92.2 (11.5)	51	91.8 (14.7)	34	105.2 (12.2)	<0.001
10-11	39	90.2 (12.1)	42	90.1 (20.1)	29	98.7 (11.8)	0.042
12-15	54	80.6 (17.0)	94	89.7 (14.2)	91	94.0 (13.9)	0.039
16-19	85	71.1 (24.6)	56	79.1 (21.8)	73	92.3 (16.1)	<0.001
20-23	84	62.2 (21.3)	84	70.9 (25.5)	51	85.9 (24.6)	<0.001
24-27	76	61.0 (25.6)	75	59.0 (25.5)	41	82.3 (24.0)	<0.001
28-31	58	58.5 (22.7)	73	62.7 (24.3)	59	63.9 (24.1)	0.771
32-35	33	60.1 (25.1)	48	67.4 (22.2)	52	73.0 (24.3)	0.239
36-39	23	59.5 (24.7)	40	54.0 (20.6)	35	78.2 (19.4)	<0.001
40-43	13	57.8 (23.5)	32	61.0 (28.7)	27	68.3 (21.8)	0.286
44-47	22	57.9 (21.7)	17	64.0 (22.8)	22	73.2 (25.2)	0.248
48-51	15	63.3 (19.9)	21	60.6 (20.9)	14	67.0 (20.0)	0.371
52-55	6	64.8 (16.7)	21	58.1 (25.6)	12	61.4 (18.7)	0.706
56-59	7	62.8 (23.6)	13	59.6 (20.4)	13	58.8 (17.8)	***
60+	6	35.3 (9.4)	13	54.5 (26.6)	21	68.3 (17.5)	0.075
<16	171	87.9 (15.3)	224	91.0 (15.9)	187	98.0 (14.6)	N/A
≥16	428	62.3 (23.8)	493	64.5 (24.9)	420	76.5 (23.9)	N/A
<18	209	86.4 (16.7)	253	90.0 (16.5)	220	97.5 (14.4)	N/A
≥18	390	60.7 (23.4)	464	63.4 (24.8)	387	74.9 (24.0)	N/A
Overall	**599**	**69.6 (24.6)**	**717**	**72.8 (25.6)**	**607**	**83.1 (24.6)**	**N/A**

* Due to missing data, means are calculated from a population of 599 in 2024, 717 in 2019 and 599 in 2014.

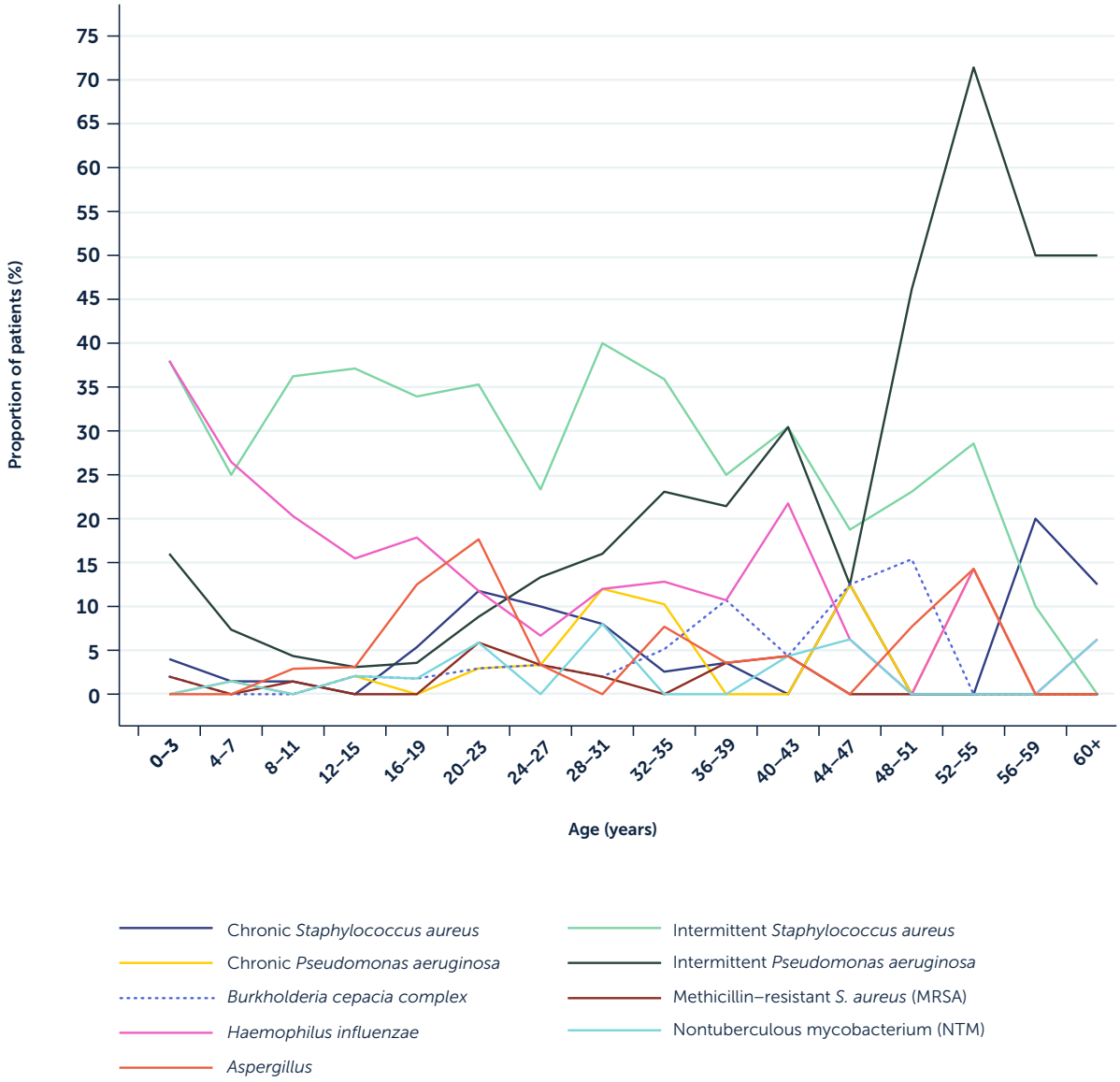
** t-test comparing 2024 with 2019. If the p-value is less than 0.05 then the difference in the mean is statistically significant.

*** t-test not performed due to small numbers in these age groups.

Lung infections

Lung infections can permanently reduce lung function in people with cystic fibrosis. Some lung infections can become 'chronic', meaning that they can't ever be removed completely using medicines. All other infections are reported if they have occurred at least once as a positive growth in the 12 months prior to the patient's annual review.

1.16 Lung infections in 2024
N=606*



* Proportions are calculated from the number of patients with at least one sample taken in the relevant age group. This is a change from the 2020 data report where they were calculated from the number of people with annual reviews in the age group.

1.16 Lung infections in 2024 (cont.)
<16 years N=284; ≥16 years N=322

Infections in this table reflect those grown in the 12 months prior to the 2024 annual review.
The UK CF Registry definition of 'chronic' is three or more isolates in the last 12 months.

	Paediatric Age Range (Years)				Overall
	0-3	4-7	8-11	12-15	Paediatric (<16 years)
Number in age range	50	68	69	98	285
Number who had culture taken*	50	68	69	97	284
Chronic *S. aureus* n(%)	<5	<5	<5	0	<5
Intermittent *S. aureus* n(%)	19 (38.0)	17 (25.0)	25 (36.2)	36 (37.1)	97 (34.2)
Chronic *P. aeruginosa* n(%)	0	<5	0	<5	<5
Intermittent *P. aeruginosa* n(%)	8 (16.0)	5 (7.4)	<5	<5	19 (6.7)
B. cepacia complex n(%)	<5	0	0	<5	<5
B. cenocepacia n(%)	0	0	0	0	0
B. multivorans n(%)	0	0	0	0	0
B. other cepacia n(%)	<5	0	0	<5	<5
MRSA n(%)	<5	0	<5	0	<5
H. influenza n(%)	19 (38.0)	18 (26.5)	14 (20.3)	15 (15.5)	66 (23.2)
NTM n(%)	0	<5	0	<5	<5
Aspergillus n(%)	0	0	<5	<5	5 (1.8)

* Proportions are calculated from the number of people who were recorded as having at least one respiratory culture sample taken.

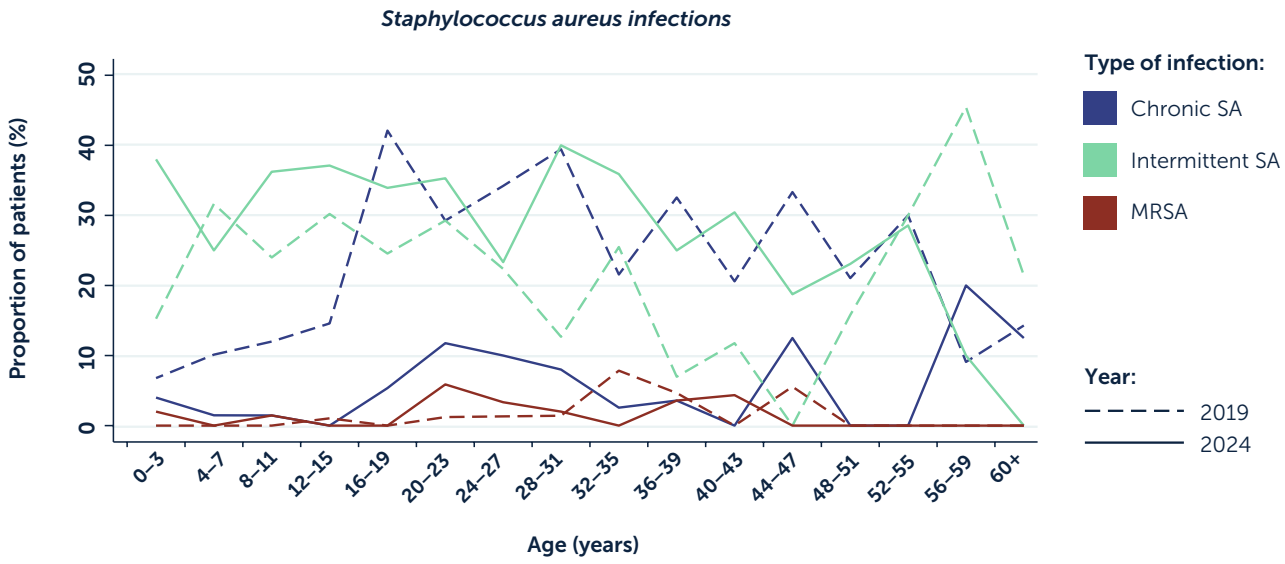
1.16 Lung infections in 2024 (cont.)
<16 years N=284; ≥16 years N=322

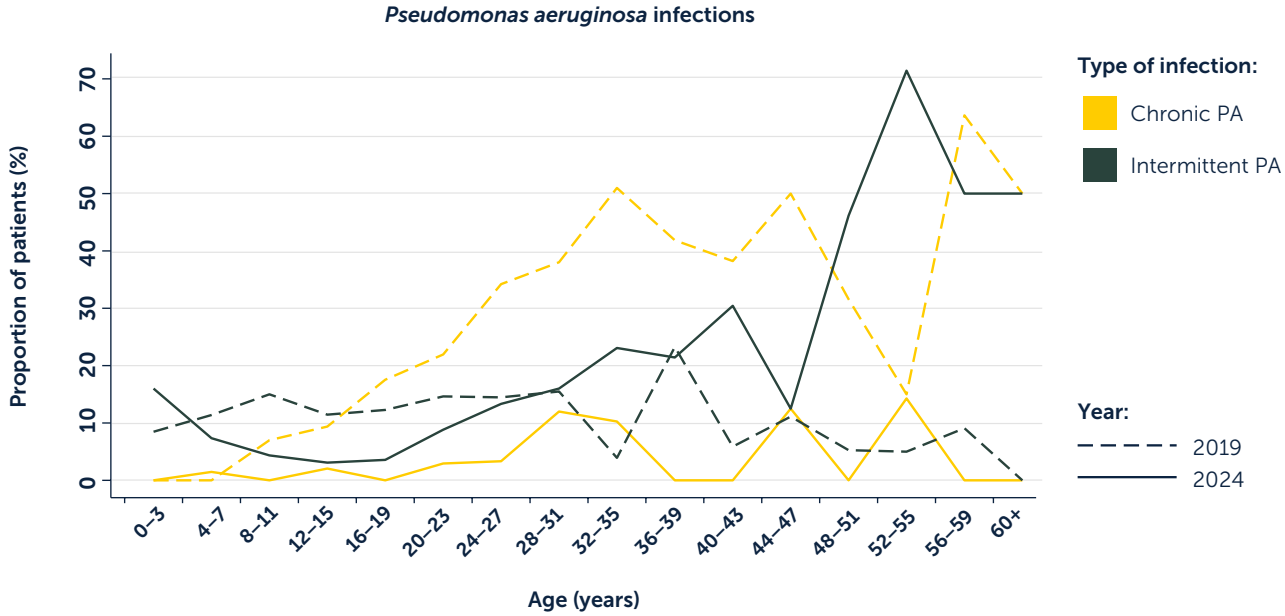
	Adult Age Range (Years)						Overall
	16-19	20-23	24-27	28-31	32-35	36-39	Adults (≥16 years)
Number in age range	74	52	42	60	55	37	446
Number who had culture taken*	56	34	30	50	39	28	322
Chronic *S. aureus* n(%)	<5	<5	<5	<5	<5	<5	22 (6.8)
Intermittent *S. aureus* n(%)	19 (33.9)	12 (35.3)	7 (23.3)	20 (40.0)	14 (35.9)	7 (25.0)	95 (29.5)
Chronic *P. aeruginosa* n(%)	0	<5	<5	6 (12.0)	<5	0	15 (4.7)
Intermittent *P. aeruginosa* n(%)	<5	<5	<5	8 (16.0)	9 (23.1)	6 (21.4)	65 (20.2)
B. cepacia complex n(%)	<5	<5	<5	<5	<5	<5	14 (4.3)
B. cenocepacia n(%)	0	0	<5	0	<5	<5	5 (1.6)
B. multivorans n(%)	<5	0	0	0	0	<5	5 (1.6)
B. other cepacia n(%)	0	<5	0	0	0	0	<5
MRSA n(%)	0	<5	<5	<5	0	<5	6 (1.9)
H. influenza n(%)	10 (17.9)	<5	<5	6 (12.0)	5 (12.8)	<5	38 (11.8)
NTM n(%)	<5	<5	0	<5	0	0	10 (3.1)
Aspergillus n(%)	7 (12.5)	6 (17.6)	<5	0	<5	<5	21 (6.5)

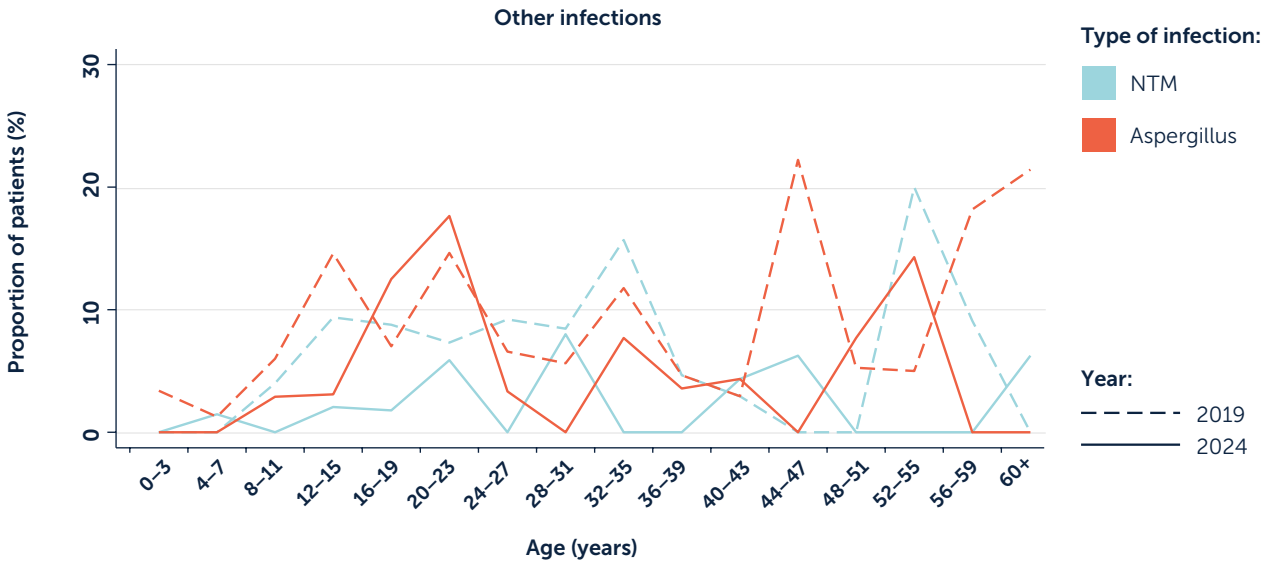
	Adult Age Range (Years)						Overall
	40-43	44-47	48-51	52-55	56-59	60-63	Adults (≥16 years)
Number in age range	31	28	17	13	14	23	446
Number who had culture taken*	23	16	13	7	10	16	322
Chronic *S. aureus* n(%)	0	<5	0	0	<5	<5	22 (6.8)
Intermittent *S. aureus* n(%)	7 (30.4)	<5	<5	<5	<5	0	95 (29.5)
Chronic *P. aeruginosa* n(%)	0	<5	0	<5	0	0	15 (4.7)
Intermittent *P. aeruginosa* n(%)	7 (30.4)	<5	6 (46.2)	5 (71.4)	5 (50.0)	8 (50.0)	65 (20.2)
B. cepacia complex n(%)	<5	<5	<5	0	0	0	14 (4.3)
B. cenocepacia n(%)	<5	0	<5	0	0	0	5 (1.6)
B. multivorans n(%)	0	<5	0	0	0	0	5 (1.6)
B. other cepacia n(%)	0	0	0	0	0	0	<5
MRSA n(%)	<5	0	0	0	0	0	6 (1.9)
H. influenza n(%)	5 (21.7)	<5	0	<5	0	<5	38 (11.8)
NTM n(%)	<5	<5	0	0	0	<5	10 (3.1)
Aspergillus n(%)	<5	0	<5	<5	0	0	21 (6.5)

* Proportions are calculated from the number of people who were recorded as having at least one respiratory culture sample taken.

1.17 Lung infections 2019 – 2024







1.18 Respiratory culture sample type[1]

Overall	2019	2024
Number of people with an annual review (N)	868	731
Number of people with at least 3 sample of any type taken N(%)*	697 (80.3)	423 (57.9)
Number of people with at least 1 sample of any type taken N(%)*	831 (95.7)	607 (83.0)
Sample type[1]**		
Sputum; N(%)	609 (73.3)	341 (56.2)
Cough; N(%)	421 (50.7)	414 (68.2)
Bronchoalveolar lavage; N(%)	25 (3.0)	11 (1.8)
Age <16 years	**2019**	**2024**
Number of people with an annual review (N)	336	285
Number of people with at least 3 sample of any type taken N(%)*	306 (91.1)	269 (94.4)
Number of people with at least 1 sample of any type taken N(%)*	335 (99.7)	284 (99.6)
Sample type[1]**		
Sputum; N(%)	129 (38.5)	60 (21.1)
Cough; N(%)	326 (97.3)	284 (100.0)
Bronchoalveolar lavage; N(%)	19 (5.7)	6 (2.1)
Age ≥16 years	**2019**	**2024**
Number of people with an annual review (N)	532	446
Number of people with at least 3 sample of any type taken N(%)*	391 (73.5)	154 (34.5)
Number of people with at least 1 sample of any type taken N(%)*	496 (93.2)	323 (72.4)
Sample type[1]**		
Sputum; N(%)	480 (96.8)	281 (87.0)
Cough; N(%)	95 (19.2)	130 (40.2)
Bronchoalveolar lavage; N(%)	6 (1.2)	5 (1.5)

* % is of those people with an annual review.

** Patients can have more than one sample taken so the % total may not add up to 100%.

1 Proportions are calculated from the number of people with at least 1 sample of any type taken.

1.19 Non-tuberculous mycobacteria (NTM) or atypical mycobacteria

Non-tuberculous mycobacterium is slow to grow and takes time to treat. It may be present for several years before eradication or may never be cleared. In the table below, prevalence represents all people reported in that year as having a positive culture. Incidence represents all positive cultures in individuals that have not reported having any in the previous two years of data.

	2022	2023	2024
Number with annual review	**(n=727)**	**(n=723)**	**(n=731)**
NTM Prevalence; n(%)	24 (3.3)	27 (3.7)	13 (1.8)
On NTM treatment in the given year; n (% of NTM prevalence in given year)	5 (20.8)	10 (37.0)	5 (38.5)
NTM Incidence[1]	17 (2.5)	18 (2.6)	7 (1.0)
M. abscessus prevalence	<5	5 (0.7)	<5
M. abscessus incidence[2]	<5	<5	<5

1 Proportion based on the number of patients with non-positive NTM tests in the previous two data years

2 Proportion based on the number of patients with non-positive *M.abscessus* tests in the previous two data years

Complications

1.20a Complications 2019 and 2024 in young people (<16 years)

The number shown is for a complication that has been present in the 12 months prior to the 2019 and 2024 annual reviews respectively. Proportions are from all people younger than 16 years at annual review.[1]

Complications	2019 (N=336)	2024 (N=285)
Respiratory related		
Nasal polyps*	5 (1.5)	<5
Sinus disease	<5	0
Asthma	<5	0
ABPA	8 (2.4)	0
Haemoptysis (massive, severe and/or moderate)	0	0
Massive haemoptysis	0	0
Pneumothorax requiring chest tube	0	0
Cardiac complications		
Tachyarrhythmia	0	0
Bradycardia	0	0
Cardiac arrest	0	0
Cardiomyopathy	0	0
Congenital heart disease	0	0
Heart failure	0	0
Ischaemic heart disease	0	0
Valvular disease	0	0
Other	0	0
Pancreas and hepatobiliary disease		
Raised liver enzymes	22 (6.5)	9 (3.2)
Liver disease	33 (9.8)	32 (11.2)
Cirrhosis with no portal hypertension	<5	0
Cirrhosis with portal hypertension	5 (1.5)	<5
Gall bladder disease requiring surgery	<5	5 (1.8)
Pancreatitis	<5	0
Upper gastrointestinal (GI)		
Gastro-oesphageal reflux disease (GORD)	<5	0
Peptic ulcer	0	0
GI bleed (varices as source)	<5	0

* Previous reports listed this complication as "Nasal polyps requiring surgery". The UK CF Registry does not specify a difference between nasal polyps and nasal polyps requiring surgery therefore we have changed the wording.

1.20a Complications 2019 and 2024 in young people (<16 years) (cont.)

Complications	2019 (N=336)	2024 (N=285)
GI bleed (non varices as source)	0	0
Lower gastrointestinal		
Intestinal obstruction	<5	0
DIOS	7 (2.1)	6 (2.1)
Fibrosing colonopathy / colonic stricture	0	0
Rectal prolapse	0	<5
Renal		
Kidney stones	<5	0
Renal failure	0	<5
Musculoskeletal		
Arthritis	<5	<5
Arthropathy	<5	0
Bone fracture	0	<5
Osteopenia	0	<5
Osteoporosis	0	0
Other		
Cancer confirmed by histology	0	0
Port inserted or replaced	6 (1.8)	0
Depression	0	0
Hearing loss	<5	<5
Hypertension	0	0
Urinary incontinence	<5	<5
Faecal incontinence	<5	<5
Postural anomaly	<5	<5

1.20b Complications 2019 and 2024 in adults (ages 16 years and older)

The number shown is for a complication that has been present in the 12 months prior to the 2019 and 2024 annual reviews respectively. Proportions are from all people ages 16 years and older at annual review.[1]

Complications	2019 (N=532)	2024 (N=446)
Respiratory related		
Nasal polyps*	21 (3.9)	7 (1.6)
Sinus disease	103 (19.4)	52 (11.7)
Asthma	46 (8.6)	51 (11.4)
ABPA	31 (5.8)	14 (3.1)
Any haemoptysis	15 (2.8)	6 (1.3)
Haemoptysis (massive, severe and/or moderate)	<5	<5
Pneumothorax requiring chest tube	<5	0
Cardiac complications		
Tachyarrhythmia	0	0
Bradycardia	0	0
Cardiac arrest	0	<5
Cardiomyopathy	<5	<5
Congenital heart disease	0	0
Heart failure	0	<5
Ischaemic heart disease	<5	0
Valvular disease	<5	0
Other	<5	0
Pancreas and hepatobiliary disease		
Raised liver enzymes	39 (7.3)	33 (7.4)
Liver disease	100 (18.8)	114 (25.6)
Cirrhosis with no portal hypertension	7 (1.3)	6 (1.3)
Cirrhosis with portal hypertension	10 (1.9)	14 (3.1)
Gall bladder disease requiring surgery	<5	<5
Pancreatitis	6 (1.1)	8 (1.8)
Upper gastrointestinal (GI)		
Gastro-oesphageal reflux disease (GORD)	147 (27.6)	181 (40.6)
Peptic ulcer	0	0
GI bleed (varices as source)	0	0
GI bleed (non varices as source)	<5	<5

* Previous reports listed this complication as "Nasal polyps requiring surgery". The UK CF Registry does not specify a difference between nasal polyps and nasal polyps requiring surgery therefore we have changed the wording.

1.20b Complications 2019 and 2024 in adults (ages 16 years and older) (cont.)

Complications	2019 (N=532)	2024 (N=446)
Lower gastrointestinal		
Intestinal obstruction	<5	<5
DIOS	80 (15.0)	37 (8.3)
Fibrosing colonopathy / colonic stricture	0	0
Rectal prolapse	0	0
Renal		
Kidney stones	<5	0
Renal failure	8 (1.5)	<5
Musculoskeletal		
Arthritis	6 (1.1)	6 (1.3)
Arthropathy	38 (7.1)	9 (2.0)
Bone fracture	<5	<5
Osteopenia	89 (16.7)	95 (21.3)
Osteoporosis	33 (6.2)	39 (8.7)
Other		
Cancer confirmed by histology	0	<5
Port inserted or replaced	6 (1.1)	<5
Depression	23 (4.3)	12 (2.7)
Hearing loss	13 (2.4)	12 (2.7)
Hypertension	10 (1.9)	12 (2.7)
Urinary incontinence	55 (10.3)	21 (4.7)
Faecal incontinence	<5	<5
Postural Anomaly	48 (9.0)	11 (2.5)

1.21 Incidence of complications

The table below describes new cases of a complication that have not been reported for an individual in at least the previous two years.

	Overall (n=731)	<16 years (n=285)	≥16 years (n=446)
ABPA	11 (1.5)	0	11 (2.5)
Cirrhosis - no portal hypertension	<5	0	<5
Cirrhosis - with portal hypertension	5 (0.7)	<5	<5
Cancer confirmed by histology	0	0	0

1.22 CF diabetes**
N=575

Cystic fibrosis diabetes (CFD) is common in adults and adolescents with cystic fibrosis. This is because, for many people with CF, the pancreas does not work properly. This can mean that not enough insulin is produced, or it may not work properly, causing CFD. CFD is different from type 1 and type 2 diabetes, but has features of both.

	All ≥10 years (575)	10-15 years (129)	≥16 years (n=446)
On CFD treatment			
People on CFD treatment; n (%)	92 (16.0)	11 (8.5)	81 (18.2)
Insulin; n(%)[1]	87 (94.6)	11 (100.0)	76 (93.8)
CFRD Screening ; n(%)			
Yes	291 (50.6)	91 (70.5)	200 (44.8)
Screening Type			
Continous glucose monitoring; n(%)[2]	94 (32.3)	38 (41.8)	56 (28.0)
Oral glucose tolerance test; n(%)[2]	276 (94.8)	68 (74.7)	208 (104.0)
Not screened (known CFD)	125 (21.7)	11 (8.5)	114 (25.6)
Not screened (other)	155 (27.0)	27 (20.9)	128 (28.7)
Unknown	<5	0	<5

1 Proportion of patients on treatment.
2 Proportion of patients screened.
* Redacted to adhere to statistical disclosure guidelines.
** Alternatively known as CF related diabetes.

Antibiotics

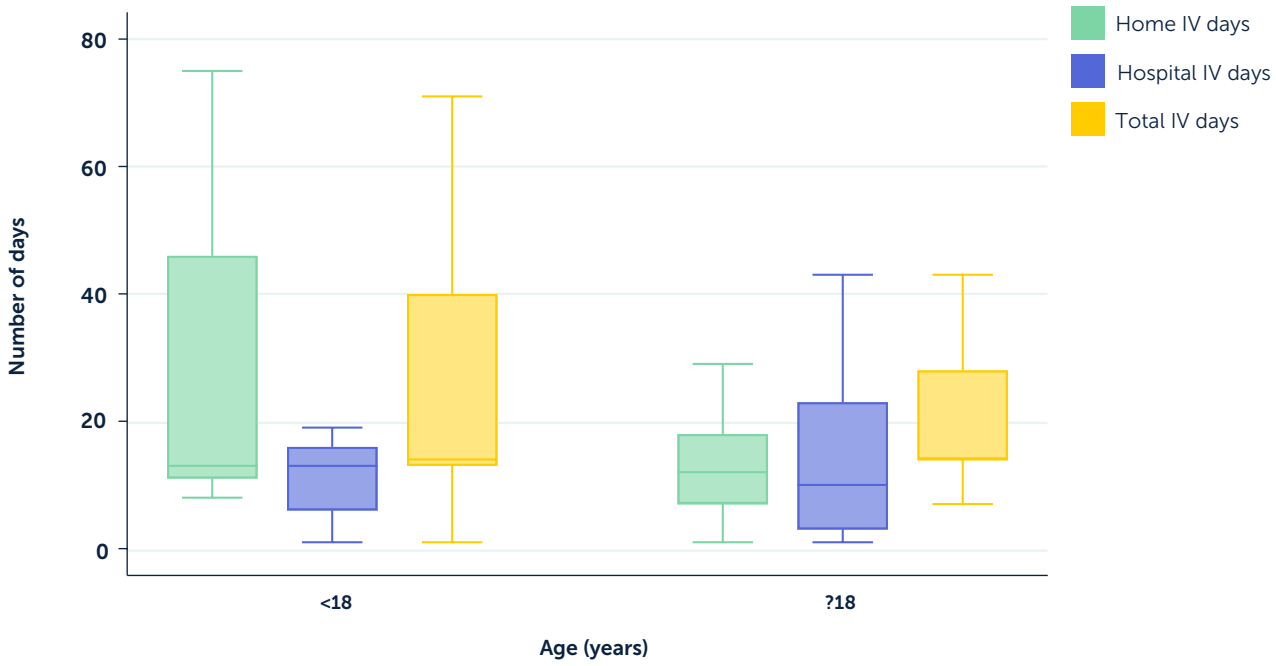
1.23 Intravenous (IV) antibiotics
N=731

When someone with CF becomes unwell with an infection, they might be prescribed intravenous (IV) antibiotics. This treatment can take a number of days and may take place as a hospital inpatient, or at home.

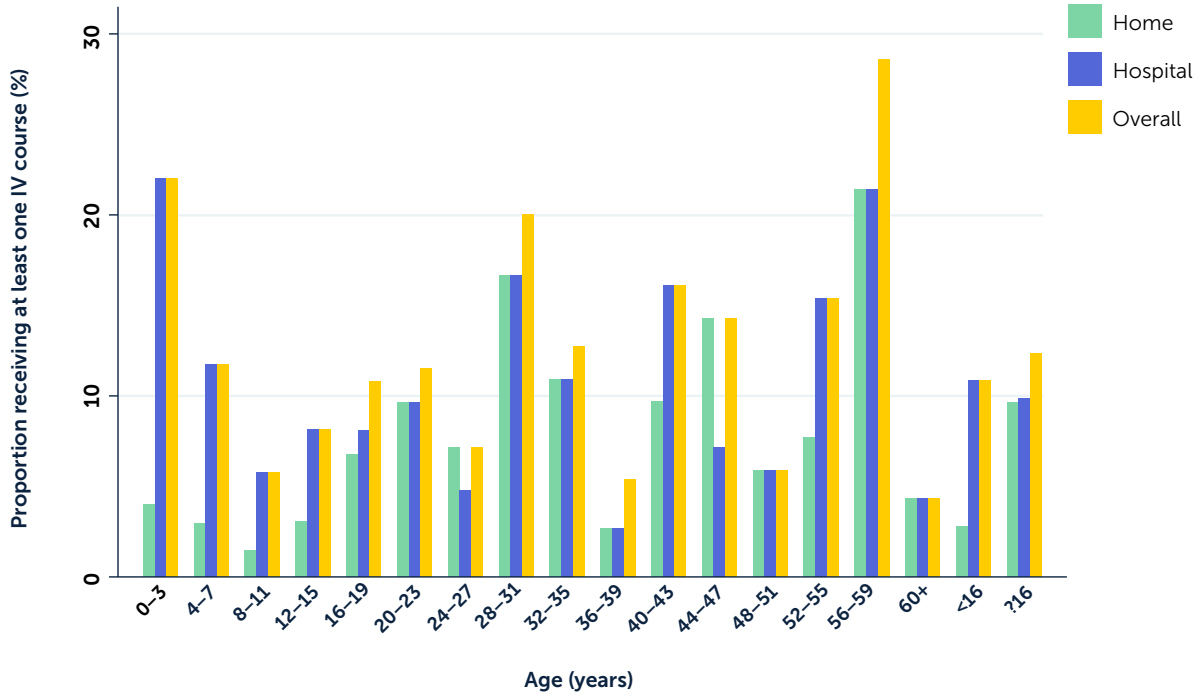
		Home		Hospital		Total	
Age	n	Patients n(%)	Median days (IQR)	Patients n(%)	Median days (IQR)	Patients n(%)	Median days (IQR)
0-3	50	<5	*	11 (22.0)	14 (5-17)	11 (22.0)	16 (13-19)
4-7	68	<5	*	8 (11.8)	14 (11-28)	8 (11.8)	14 (14-42)
8-11	69	<5	*	<5	*	<5	*
12-15	98	<5	*	8 (8.2)	14 (4-40)	8 (8.2)	14 (14-61)
16-19	74	5 (6.8)	12 (12-18)	6 (8.1)	9 (2-12)	8 (10.8)	14 (13-18)
20-23	52	5 (9.6)	12 (8-14)	5 (9.6)	25 (23-27)	6 (11.5)	32 (14-36)
24-27	42	<5	*	<5	*	<5	*
28-31	60	10 (16.7)	16 (4-37)	10 (16.7)	12 (5-24)	12 (20.0)	28 (14-49)
32-35	55	6 (10.9)	10 (6-11)	6 (10.9)	7 (3-36)	7 (12.7)	14 (13-42)
36-39	37	<5	*	<5	*	<5	*
40-43	31	<5	*	5 (16.1)	5 (4-14)	5 (16.1)	14 (14-21)
44-47	28	<5	*	<5	*	<5	*
48-51	17	<5	*	<5	*	<5	*
52-55	13	<5	*	<5	*	<5	*
56-59	14	<5	*	<5	*	<5	*
60+	23	<5	*	<5	*	<5	*
<16	285	8 (2.8)	13 (11-46)	31 (10.9)	14 (5-17)	31 (10.9)	14 (13-41)
≥16	446	43 (9.6)	12 (7-18)	44 (9.9)	10 (3-22)	55 (12.3)	14 (14-28)
<18	319	8 (2.5)	13 (11-46)	33 (10.3)	13 (6-16)	33 (10.3)	14 (13-40)
≥18	412	43 (10.4)	12 (7-18)	42 (10.2)	10 (3-23)	53 (12.9)	14 (14-28)
Overall	**731**	**51 (7.0)**	**12 (8-22)**	**75 (10.3)**	**12 (4-19)**	**86 (11.8)**	**14 (14-28)**

* Summary statistics not provided for very small samples.

This box plot graph illustrates the spread of the number of days on IV antibiotics in the Scottish CF population, stratified by age. A guide on how to correctly interpret this box plot graph can be found on page 56.



The bar graph below summarises the proportion of people receiving at least one course of IV antibiotics across different age groups within the Scottish CF population. Overall, the proportion of patients receiving at least one IV course at home was 8.6% and in hospital was 17.0%. The proportion receiving any IVs was 20.0%.



1.24 Inhaled antibiotic use
N=731

	2024		
	Overall	<16 years	≥16 years
Number of patients	731	285	446
Tobramycin solution; n(%)	74 (10.1)	24 (8.4)	50 (11.2)
Colistin; n(%)	151 (20.7)	71 (24.9)	80 (17.9)
Promixin; n(%)	43 (5.9)	8 (2.8)	35 (7.8)
Aztreonam; n(%)	26 (3.6)	<5	-*
Colistimethate (DPI); n(%)	72 (9.8)	8 (2.8)	64 (14.3)
Tobramycin Inhalation Powder; n(%)	82 (11.2)	<5	-*
Levofloxacin; n(%)	8 (1.1)	0	8 (1.8)
At least one of the above; n(%)	284 (38.9)	87 (30.5)	197 (44.2)

1.25 Inhaled antibiotic use among people with chronic *Pseudomonas aeruginosa*

The consensus view in the UK is that 90% of people chronically infected with *Pseudomonas aeruginosa* should be prescribed at least one of the above inhaled antibiotics.

	2024
	All ages**
Patients with chronic *P. aeruginosa*	18
Tobramycin solution; n(%)	6 (33.3)
Other aminoglycoside; n(%)	0
Colistin; n(%)	6 (33.3)
Promixin; n(%)	<5
Aztreonam; n(%)	5 (27.8)
Colistimethate (DPI); n(%)	5 (27.8)
Tobramycin Inhalation Powder; n(%)	6 (33.3)
Levofloxacin; n(%)	<5
At least one of the above; n(%)	15 (83.3)

* Redacted to adhere to statistical disclosure guidelines

** Fewer than 5 people under age 16 years has chronic *Pseudomonas aeringinosa*

1.26 Long-term azithromycin use

Azithromycin is an antibiotic with some anti-inflammatory properties. It is recommended for long-term use as a prophylactic antibiotic in people with chronic *Pseudomonas aeruginosa*.

		Number of patients on azithromycin; n	Patients with chronic *P. aeruginosa*; n(%)	Patients without chronic *P. aeruginosa*; n(%)
2014	Overall	404	187 (46.3)	217 (53.7)
	0-3 years	-*	<5	<5
	4-15 years	-*	-*	55 (85.9)
	≥ 16 years	337	177 (52.5)	160 (47.5)
2019	Overall	454	163 (35.9)	291 (64.1)
	0-3 years	7	0	7 (100.0)
	4-15 years	84	11 (13.1)	73 (86.9)
	≥ 16 years	363	152 (41.9)	211 (58.1)
2024	Overall	267	17 (6.4)	250 (93.6)
	0-3 years	-*	-*	-*
	4-15 years	-*	<5	-*
	≥ 16 years	200	15 (7.5)	185 (92.5)

1.27 Flucloxacillin use

Flucloxacillin is an antibiotic that is used prophylactically to prevent infection with bacteria.

	2019		2024	
Age	Total patients	Patients on prophylactic flucloxacillin; n(%)	Total patients	Patients on prophylactic flucloxacillin; n(%)
0-3	60	39 (65.0)	50	34 (68.0)
4-7	79	31 (39.2)	68	46 (67.6)
8-11	100	37 (37.0)	69	43 (62.3)
12-15	97	32 (33.0)	98	56 (57.1)
16-19	58	12 (20.7)	74	16 (21.6)
20-23	86	28 (32.6)	52	5 (9.6)
24-27	77	13 (16.9)	42	6 (14.3)
28-31	78	10 (12.8)	60	9 (15.0)
32-35	54	<5	55	8 (14.5)
36-39	47	<5	37	<5
40-43	37	<5	31	0
44-47	22	0	28	0
48-51	21	<5	17	<5
52-55	23	<5	13	<5
56-59	14	<5	14	0
60+	15	0	23	0
<16 years	336	139 (41.4)	285	179 (62.8)
≥16 years	532	76 (14.3)	446	50 (11.2)
<18 years	366	148 (40.4)	319	187 (58.6)
≥18 years	502	67 (13.3)	412	42 (10.2)
Overall	**868**	**215 (24.8)**	**731**	**229 (31.3)**

* Redacted to adhere to statistical disclosure guidelines.

** Data incudes patients that have been recruited and randomised for the CF START trial from 2018–2023. The CF START trial is investigating the use of prophylactic flucloxacillin use in infants. You can learn more about this trial here: **https://cfstart.org.uk/**

Bronchodilators and corticosteroids

1.28 Inhaled bronchodilators and corticosteroids

Age	Total patients	Patients on inhaled bronchodilators; n(%)	Patients on inhaled corticosteroids; n(%)	Patients on inhaled combination corticosteroids/ bronchodilators; n(%)
<6 years	83	12 (14.5)	7 (8.4)	<5
6 - ≤16 years	217	53 (24.4)	31 (14.3)	12 (5.5)
6 - ≤18 years	256	64 (25.0)	35 (13.7)	13 (5.1)
<16 years	285	63 (22.1)	36 (12.6)	12 (4.2)
≥16 years	446	267 (59.9)	92 (20.6)	64 (14.3)
<18 years	319	70 (21.9)	40 (12.5)	14 (4.4)
≥18 years	412	260 (63.1)	88 (21.4)	62 (15.0)
Overall	**731**	**330 (45.1)**	**128 (17.5)**	**76 (10.4)**

Muco-active therapies

1.29 Mannitol

	2019		2024	
Age	Total patients	Patients on Mannitol; n (%)	Total patients	Patients on Mannitol; n (%)
<16 years	336	0	285	0
≥16 years	532	11 (2.1)	446	9 (2.0)
<18 years	366	0	319	0
≥18 years	502	11 (2.2)	412	9 (2.2)
Overall	**868**	**11 (1.3)**	**731**	**9 (1.2)**

1.30 DNase**

	2014		2019		2024	
Age	Total patients	Patients on DNase; n(%)	Total patients	Patients on DNase; n(%)	Total patients	Patients on DNase; n(%)
0-3	74	5 (6.8)	60	6 (10.0)	50	7 (14.0)
4-7	79	18 (22.8)	79	17 (21.5)	68	19 (27.9)
8-11	89	31 (34.8)	100	42 (42.0)	69	29 (42.0)
12-15	55	28 (50.9)	97	52 (53.6)	98	57 (58.2)
16-19	89	38 (42.7)	58	31 (53.4)	74	50 (67.6)
20-23	88	35 (39.8)	86	51 (59.3)	52	31 (59.6)
24-27	82	41 (50.0)	77	42 (54.5)	42	31 (73.8)
28-31	67	21 (31.3)	78	49 (62.8)	60	40 (66.7)
32-35	46	8 (17.4)	54	24 (44.4)	55	42 (76.4)
36-39	29	9 (31.0)	47	22 (46.8)	37	20 (54.1)
40-43	18	<5	37	14 (37.8)	31	19 (61.3)
44-47	24	9 (37.5)	22	9 (40.9)	28	13 (46.4)
48-51	19	6 (31.6)	21	9 (42.9)	17	11 (64.7)
52-55	7	<5	23	9 (39.1)	13	7 (53.8)
56-59	7	0	14	6 (42.9)	14	7 (50.0)
60+	9	<5	15	<5	23	11 (47.8)
<16 years	297	82 (27.6)	336	117 (34.8)	285	112 (39.3)
≥16 years	485	174 (35.9)	532	270 (50.8)	446	282 (63.2)
<18 years	335	98 (29.3)	366	134 (36.6)	319	137 (42.9)
≥18 years	447	158 (35.3)	502	253 (50.4)	412	257 (62.4)
Overall	**782**	**256 (32.7)**	**868**	**387 (44.6)**	**731**	**394 (53.9)**

* Redacted to adhere to statistical disclosure guidelines.

** CF STORM, a clinical trial investigating the withdrawal of mucoactive therapies, was enrolling patients during the 2024 data collection year. See **www.cfstorm.org.uk** for more information.

1.31 Hypertonic saline*

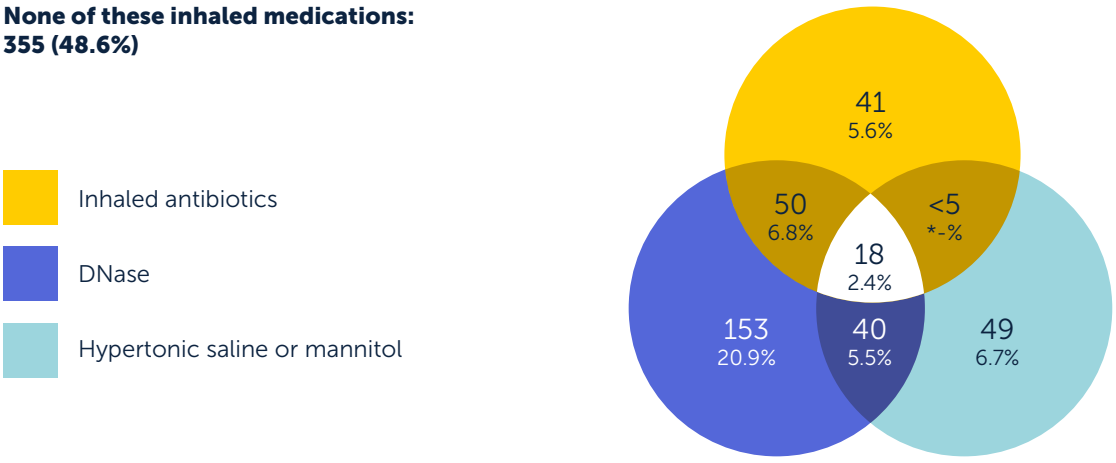
This treatment helps to thin mucus so that it is easier to cough out of the body.

	2014		2019		2024	
Age	Total patients	Patients on hypertonic saline; n(%)	Total patients	Patients on hypertonic saline; n(%)	Total patients	Patients on hypertonic saline; n(%)
0-3	74	<5	60	10 (16.7)	50	7 (14.0)
4-7	79	-*	79	14 (17.7)	68	18 (26.5)
8-11	89	14 (15.7)	100	27 (27.0)	69	24 (34.8)
12-15	55	15 (27.3)	97	38 (39.2)	98	37 (37.8)
16-19	89	28 (31.5)	58	17 (29.3)	74	27 (36.5)
20-23	88	16 (18.2)	86	18 (20.9)	52	10 (19.2)
24-27	82	15 (18.3)	77	19 (24.7)	42	10 (23.8)
28-31	67	14 (20.9)	78	11 (14.1)	60	17 (28.3)
32-35	46	<5	54	10 (18.5)	55	13 (23.6)
36-39	29	<5	47	8 (17.0)	37	6 (16.2)
40-43	18	0	37	<5	31	5 (16.1)
44-47	24	5 (20.8)	22	<5	28	<5
48-51	19	<5	21	<5	17	<5
52-55	7	0	23	<5	13	<5
56-59	7	<5	14	<5	14	<5
60+	9	<5	15	<5	23	5 (21.7)
<16 years	297	41 (13.8)	336	89 (26.5)	285	86 (30.2)
≥16 years	485	93 (19.2)	532	94 (17.7)	446	102 (22.9)
<18 years	335	50 (14.9)	366	98 (26.8)	319	103 (32.3)
≥18 years	447	84 (18.8)	502	85 (16.9)	412	85 (20.6)
Overall	**782**	**134 (17.1)**	**868**	**183 (21.1)**	**731**	**188 (25.7)**

1.32 Burden of treatment

The Venn diagram shows how many people with CF are on one or more inhaled therapies and the combinations they take. A total of 355 (48.6%) people in Scotland are on no inhaled therapies.

None of these inhaled medications: 355 (48.6%)



* CF STORM, a clinical trial investigating the withdrawal of mucoactive therapies, was enrolling patients during the 2024 data collection year. See **www.cfstorm.org.uk** for more information

CFTR modulators

Here we describe the eligibility for and access to CFTR modulators throughout[1] 2024, including the NHS agreement in mid-2024 for long-term access to CFTR modulators. The access arrangements prior to 2024 are described in previous annual reports.

Elexacaftor/tezacaftor/ivacaftor

During 2024, elexacaftor/tezacaftor/ivacaftor (ETI) was available in the UK for patients with cystic fibrosis aged 2 years and over who have at least one copy of the F508del variant. Guidance was issued throughout the year from NHS commissioners across the devolved nations to support the prescribing of ETI "off-label". This varies slightly across the devolved nations but covers the 178 variants that are on an approved FDA list and the 7 named variants in the French Compassionate Use Programme. Individuals with at least one copy of one of the variants on the approved list were potentially eligible for ETI.

Lumacaftor/ivacaftor

Lumacaftor/ivacaftor is licensed for use in the UK for patients aged one and over with two copies of the F508del mutation.

Ivacaftor

Ivacaftor is licensed for use for people aged four months and older with at least one copy of a CFTR gating mutation, one copy of the R117H gene, or one copy of one of the 97 named variants on the approved FDA list[2], which are mutations considered responsive to ivacaftor monotherapy based on in-vitro and clinical data.

Tezacaftor/ivacaftor

Tezacaftor/ivacaftor is licensed for use in patients aged six and over who have two copies of the F508del mutation, or a single copy of F508del and one of 14 residual function mutations[3]. Tezacaftor/ivacaftor is also available for use in patients aged six and over who have at least one copy of one of the 154 named variants on the approved FDA list[4], which are mutations considered responsive to tezacaftor/ivacaftor combination therapy based on in-vitro and clinical data.

1 **https://www.cysticfibrosis.org.uk/the-work-we-do/campaigning-hard/life-saving-drugs/nice-modulator-appraisal**. Note: In December 2024, access expanded to 272 named variants. The additional 94 variants named in this expansion are not considered in this report as "eligible" to access Kaftrio as this change was made at the very end of the 2024 annual review year.

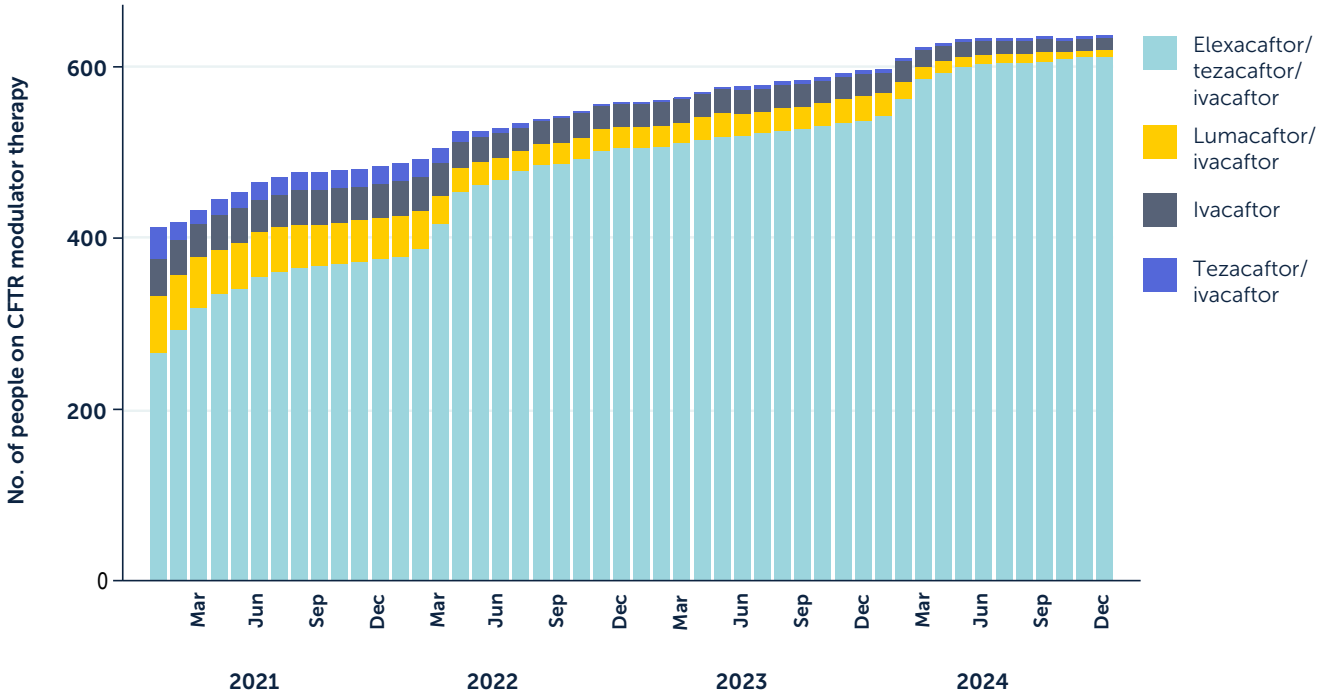
2 **https://pi.vrtx.com/files/uspi_ivacaftor.pdf**

3 **https://www.cysticfibrosis.org.uk/what-is-cystic-fibrosis/cystic-fibrosis-care/treatments-and-medication/symkevi**

4 **https://pi.vrtx.com/files/uspi_tezacaftor_ivacaftor.pdf**

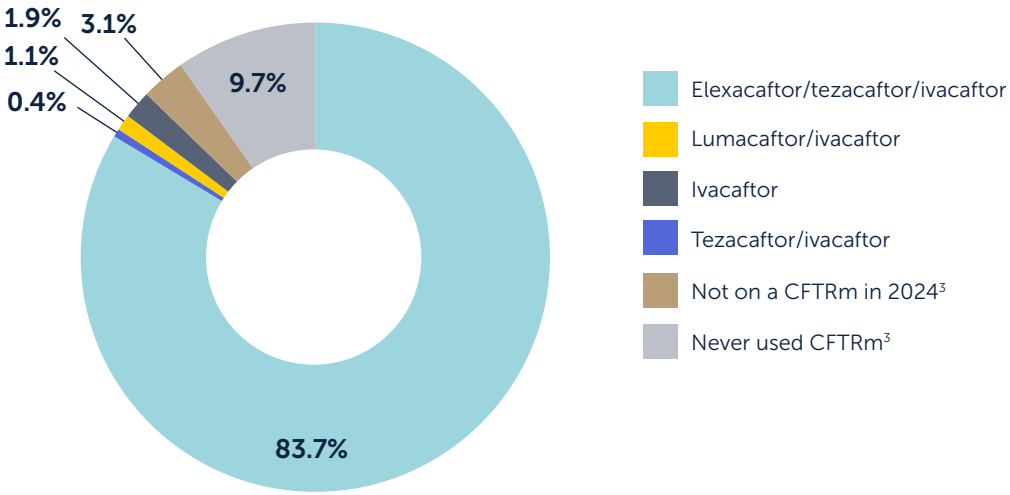
1.33 CFTR modulator uptake 2021 – 2024 among people with a 2024 annual review[1]
N=731

The graph below shows the number of people taking each drug by month. Where people switched modulators, the most recent prescription is counted. Only patients who had an annual review are counted.



1.34a CFTR modulator use in 2024
N=731[2]

This chart shows the distribution of CFTR modulators taken during 2024. Where an individual had multiple prescriptions, only the most recent one was counted. 630 people had a record of using a CFTRm in 2024.



1 All people of all ages with a 2024 annual review are included.

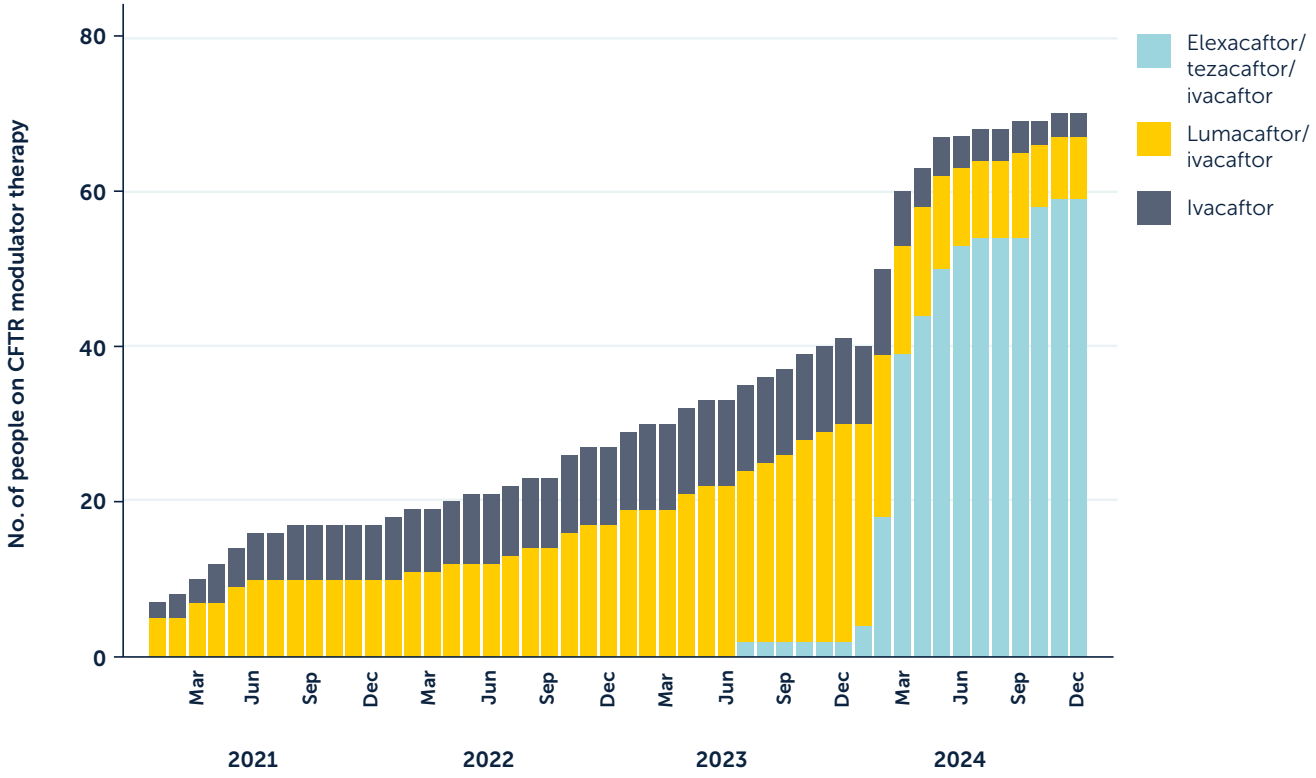
2 10 people were excluded because their last prescribed CFTRm treatment was through a clinical trial and specific treatment was unknown.

3 An individual was categorized as 'Not on a CFTRm in 2024' if they had a previous record of CFTRm use but no record of CFTRm use in 2024. Individuals categorized as 'Never used CFTRm' had no records of CFTRms prior to or in 2024.

1.34b CFTR modulator uptake in 2021 – 2024 in people ages 6 and under

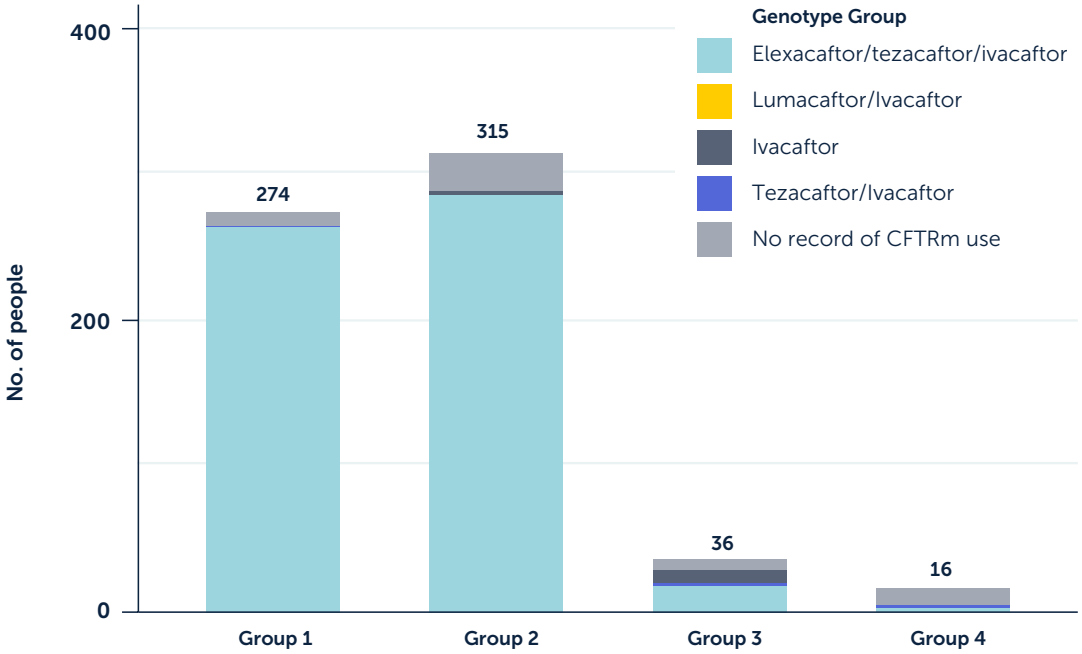
N=70[1]

The landscape of eligibility for CFTR modulator treatment has continued to expand. In 2024, elexacaftor/tezacaftor/ivacaftor, lumacaftor/ivacaftor, and ivacaftor monotherapy were available to people younger than 6 years old, with ivacaftor available for those as young as 4 months old. 80.5% of people younger than 6 years of age in 2024 have had a CFTRm record either prior to or in 2024. This graph shows the uptake of CFTR modulator use from 2021–2024 among those who were younger than 6 years on 1 January 2024.



1 All individuals included in this visual are also included in figure 1.34

1.35a CFTR modulator use in all people aged 6 years and older by genotype group [1,2]
N=644



Genotype Group Definitions	
Group 1	F508del Homozygous
Group 2	F508del Heterozygous
Group 3	No F508del, but at least one variant responsive to one of the available CFTR modulators as defined by FDA lists or French Compassionate Use Programme list (see Appendix 5 for full lists).
Group 4	No F508del and no variants from FDA and French Compassionate Use Programme lists (see Appendix 5).

		Groups			
		Group 1	Group 2	Group 3	Group 4
Most recent CFTRm used	Elexacaftor/ tezacafot/ ivacaftor	264 (96.4)	286 (90.8)	18 (50.0)	<5
	Tezacaftor/ ivacaftor	<5	0	<5	<5
	Lumacaftor/ ivacaftor	0	0	0	0
	Ivacaftor	0	<5	9 (25.0)	0
	No record of CFTRm use	-*	-*	-*	11 (68.8)
	Total	274	315	36	16

1 All individuals included in this visual are also included in figure 1.34
2 4 people were excluded because their genotype data is missing or unknown.

1.35b Demographic characteristics in all people aged 6 years and older by genotype group and CFTRm use[1,2]
N=644

	Groups 1, 2, and 3[3]		Group 4[4]
	CFTRm use recorded	no record of CFTRm use	no record of CFTRm use
Number of Individuals (n)	583	42	11
Age			
6-11 years; n(%)	106 (18.2)	<5	<5
12-17 years; n(%)	117 (20.1)	7 (16.7)	<5
18+ years; n(%)	360 (61.7)	31 (73.8)	7 (63.6)
Median years (IQR)	22 (13, 34)	36 (16, 44)	18 (12, 31)
Demographics			
Male; n (%)	308 (52.8)	20 (47.6)	6 (54.5)
In full or part time employment or school; n(% of people age 18+ years)	277 (76.9)	22 (71)	7 (100)
Ethnicity; n (%)			
White	570 (98.8)	39 (92.9)	-*
Asian	<5	<5	<5
Black	0	0	0
Mixed	<5	<5	0
Other	0	0	0
Lung Health			
FEV_1 predicted; median(IQR)	87 (67, 100)	81 (69, 104)	87 (69, 96)
Best FEV_1 pred; median(IQR)	90 (71, 102)	86 (69, 107)	87 (74, 105)
At least 1 pulmonary exacerbation since last annual review; n(%)	52 (8.9)	<5	<5
Nutritional Health			
Individuals ages 20 years and older; n	325	28	5
BMI for individuals ages 20+ years; median (IQR)	23 (19, 26)	23 (20, 29)	20 (18, 21)

1 4 people were excluded because their genotype data is missing or unknown.
2 People in groups 1, 2, and 3 for visual 1,36 are considered potentially eligible for CFTRms
3 People in group 4 for visual 1.36 are considered likely not to be eligible for CFTRms.
* Redacted to adhere to statistical disclosure guidelines

Physiotherapy

1.36 Primary Airway Clearance

Physiotherapy helps people with CF clear sticky mucus from their lungs. The listed techniques represent the primary form of physiotherapy recorded for an individual on the Registry. One primary airway clearance technique can be recorded for an individual.

	Overall (n=731)	<16 years (n=285)	≥16 years (n=446)	<18 years (n=319)	≥18 years (n=412)
Active Cycle of Breathing Techniques	55 (7.5)	<5	-*	<5	-*
Assisted autogenic drainage	84 (11.5)	5 (1.8)	79 (17.7)	12 (3.8)	72 (17.5)
Autogenic drainage	92 (12.6)	<5	-*	<5	-*
Exercise; of which:	73 (10.0)	<5	-*	10 (3.1)	63 (15.3)
Exercise listed as only airway clearance technique	51 (7.0)	<5	-*	6 (1.9)	45 (10.9)
Forced expiration	5 (0.7)	0	5 (1.1)	0	5 (1.2)
High Pressure PEP	10 (1.4)	-*	<5	-*	<5
Manual techniques (percussion over pressures vibrations)	<5	<5	0	<5	0
Oscillating PEP	49 (6.7)	35 (12.3)	14 (3.1)	39 (12.2)	10 (2.4)
PEP	282 (38.6)	207 (72.6)	75 (16.8)	219 (68.7)	63 (15.3)
Postural drainage	<5	0	<5	0	<5
VEST	<5	0	<5	0	<5
Other	26 (3.6)	16 (5.6)	10 (2.2)	16 (5.0)	10 (2.4)
None	51 (7.0)	7 (2.5)	44 (9.9)	9 (2.8)	42 (10.2)

1.37 Primary or Secondary Airway Clearance Technique

People with CF may receive more than one airway clearance technique. These techniques are not mutually exclusive and represent both primary and secondary forms of physiotherapy received by people with CF.

	Overall (n=731)	<16 years (n=285)	≥16 years (n=446)	<18 years (n=319)	≥18 years (n=412)
Active Cycle of Breathing Techniques	65 (8.9)	<5	-*	5 (1.6)	60 (14.6)
Assisted autogenic drainage	125 (17.1)	33 (11.6)	92 (20.6)	43 (13.5)	82 (19.9)
Autogenic drainage	149 (20.4)	20 (7.0)	129 (28.9)	23 (7.2)	126 (30.6)
Exercise	459 (62.8)	149 (52.3)	310 (69.5)	179 (56.1)	280 (68.0)
Forced expiration	22 (3.0)	<5	-*	<5	-*
High Pressure PEP	10 (1.4)	-*	<5	-*	<5
Manual techniques (percussion over pressures vibrations)	6 (0.8)	-*	<5	-*	<5
Oscillating PEP	63 (8.6)	39 (13.7)	24 (5.4)	46 (14.4)	17 (4.1)
PEP	315 (43.1)	215 (75.4)	100 (22.4)	229 (71.8)	86 (20.9)
Postural drainage	<5	0	<5	0	<5
VEST	<5	0	<5	0	<5
Other	137 (18.7)	114 (40.0)	23 (5.2)	119 (37.3)	18 (4.4)

* Redacted to adhere to statistical disclosure guidelines

1.38 Exercise testing

Exercise testing provides valuable information on an individual's physical abilities, which gives insights into prognosis or oxygen requirements. Physiotherapists and exercise specialists use test results to individualise exercise programmes and target specific needs. Results of exercise tests can also be motivating to the individual and can be used to set future exercise goals.

Exercise test	Overall (n=731)	<16 years (n=285)	≥16 years (n=446)	<18 years (n=319)	≥18 years (n=412)
Yes[1]	133 (15.3)	18 (5.8)	115 (20.4)	24 (6.9)	109 (20.9)
No	463 (53.1)	183 (59.2)	280 (49.7)	209 (59.7)	254 (48.7)
Not known or missing	135 (15.5)	84 (27.2)	51 (9.1)	86 (24.6)	49 (9.4)
Type of exercise test[2,3]					
CPET	13 (9.8)	-*	<5	-	<5
Shuttle test	12 (9.0)	-*	<5	-*	<5
Step test	16 (12.0)	0	16 (13.9)	<5	15 (13.8)
6 minute walk test	<5	0	<5	0	<5
Other	14 (10.5)	0	14 (12.2)	0	14 (12.8)
Missing	84 (63.2)	<5	80 (69.6)	8 (33.3)	76 (69.7)

* Redacted to adhere to statistical disclosure guidelines

1 Exercise test represents all types of testing listed including submaximal, shuttle test, 6 minute walk test, step test and other test.

2 Proportion of patients who answered 'Yes' above

3 More than one type of test can be recorded so percentages may not sum to 100%

Other therapies

1.39 Oxygen and non-invasive ventilation

	Overall (n=731)	<16 years (n=285)	≥16 years (n=446)	<18 years (n=319)	≥18 years (n=412)
Non Invasive Ventillation (NIV); n (%)	0	0	0	0	0
Any oxygen use; n (%)	20 (2.7)	6 (2.1)	14 (3.1)	7 (2.2)	13 (3.2)
Among those who had oxygen use:					
Continuously	<5	0	<5	0	<5
Nocturnal or with exertion	6 (30.0)	0	6 (42.9)	0	6 (46.2)
As required (PRN)	0	0	0	0	0
With exacerbation	11 (55.0)	6 (100.0)	5 (35.7)	-*	<5

1.40 Transplants

Lung transplantation has been available to people with CF for almost 30 years. Today the most common operation carried out is a double lung transplant, or bilateral sequential lung transplant. The following table gives information about transplant activity between 2019 and 2023. There were fewer than 5 evaluations and no transplants recorded on the Registry in 2024 and so no further data is shown. Only individuals with an annual review in the given year are considered.

	2019	2020	2021	2022	2023
Patients evaluated ; n	21	11	5	10	<5
Patients accepted ; n	10	<5	<5	<5	<5
Patients receiving transplants ; n	**5**	**0**	**<5**	**<5**	**<5**
Bilateral lung ; n	<5	0	<5	0	0
Liver ; n	<5	0	0	0	0
Other ; n	0	0	0	<5	<5

1.41 Feeding

Supplementary feeding, often using a nasogastric (via the nose) or gastrostomy (via the abdomen) tube directly to the stomach, is considered when a person with CF has poor weight gain, or progressive weight loss, despite efforts to increase oral intake. The category "Any supplemental feeding" includes the subsequent categories: oral, nasogastric tube, gastronomy tube/button, jejunal, and total parenteral nutrition, and unknown or other supplemental feeding. The subsequent categories are not mutually exclusive.

Year		Overall	<16 years	≥16 years	<18 years	≥18 years
2014	**Total; n**	**782**	**297**	**485**	**335**	**447**
	Any supplemental feeding; n(%)	175 (22.4)	52 (17.5)	123 (25.4)	57 (17.0)	118 (26.4)
	Oral; n(%)	141 (18.0)	41 (13.8)	100 (20.6)	45 (13.4)	96 (21.5)
	Nasogastric tube; n(%)	10 (1.3)	0	10 (2.1)	0	10 (2.2)
	Gastrostomy tube/Button; n(%)	40 (5.1)	14 (4.7)	26 (5.4)	15 (4.5)	25 (5.6)
	Jejunal; n(%)	0	0	0	0	0
	Total Parenteral Nutrition (TPN); n(%)	0	0	0	0	0
2019	**Total; n**	**868**	**336**	**532**	**366**	**502**
	Any supplemental feeding; n(%)	219 (25.2)	87 (25.9)	132 (24.8)	92 (25.1)	127 (25.3)
	Oral; n(%)	141 (16.2)	43 (12.8)	98 (18.4)	46 (12.6)	95 (18.9)
	Nasogastric tube; n(%)	10 (1.2)	<5	-*	<5	-*
	Gastrostomy tube/Button; n(%)	32 (3.7)	14 (4.2)	18 (3.4)	14 (3.8)	18 (3.6)
	Jejunal; n(%)	0	0	0	0	0
	Total Parenteral Nutrition (TPN); n(%)	<5	0	<5	0	<5
2024	**Total; n**	**731**	**285**	**446**	**319**	**412**
	Any supplemental feeding; n(%)	101 (13.8)	29 (10.2)	72 (16.1)	33 (10.3)	68 (16.5)
	Oral; n(%)	77 (10.5)	17 (6.0)	60 (13.5)	19 (6.0)	58 (14.1)
	Nasogastric tube; n(%)	<5	0	<5	<5	<5
	Gastrostomy tube/Button; n(%)	14 (1.9)	6 (2.1)	8 (1.8)	7 (2.2)	7 (1.7)
	Jejunal; n(%)	0	0	0	0	0
	Total Parenteral Nutrition (TPN); n(%)	0	0	0	0	0

1.42 Pancreatic enzyme supplementation

Year		Overall	<16 years	≥16 years	<18 years	≥18 years
2014	**Total; n**	**782**	**297**	**485**	**335**	**447**
	Pancreatic enzyme supplements; n(%)	641 (82.0)	238 (80.1)	403 (83.1)	269 (80.3)	372 (83.2)
2019	**Total; n**	**868**	**336**	**532**	**366**	**502**
	Pancreatic enzyme supplements; n(%)	688 (79.3)	259 (77.1)	429 (80.6)	282 (77.0)	406 (80.9)
2024	**Total; n**	**731**	**285**	**446**	**319**	**412**
	Pancreatic enzyme supplements; n(%)	557 (76.2)	214 (75.1)	343 (76.9)	240 (75.2)	317 (76.9)

* Redacted to adhere to statistical disclosure guidelines.

Genotypes*

Genotypes are part of the genetic makeup of an individual that usually control a particular characteristic, known as a phenotype. For people with CF, their genotype reveals which variants of the CF gene causes their cystic fibrosis. Everyone living with CF has two variants of the gene for CFTR; one on each allele. One is inherited from their mother, and one from their father. If both variants (or genotypes) are the same, the person is said to be homozygous. Someone who has two different variants is heterozygous.

Data completeness	n(%)
Patients genotyped with at least one mutation recorded	965 (99.6)
Patients genotyped with both mutations recorded	957 (98.8)
F508del mutations	
Homozygous F508del	404 (41.7)
Heterozygous F508del	476 (49.1)

1.43 Variant combinations in Scotland

This table shows the proportion (%) of patients with the most common mutation combinations. For example, 7.4% of the Scottish population have one copy of F508del and one copy of G551D in their genotype.

CFTR variant	F508del	R117H	G551D	G542X	621+1G->T	Other	Unknown	Total
F508del	41.7							41.7
R117H	6.4	0.0						6.4
G551D	7.4	0.1	0.2					7.7
G542X	4.9	0.2	0.1	0.1				5.3
621+1G->T	0.8	0.0	0.1	0.0	0.0			0.9
Other	29.0	0.9	1.5	0.7	0.3	4.2		36.7
Unknown	0.7	0.1	0.0	0.0	0.0	0.0	0.4	1.2
Total	**90.9**	**1.3**	**2.0**	**0.8**	**0.3**	**4.2**	**0.4**	**100.0**

* In this section, we include everyone who is registered (see table 1.1) with known genotypes.

1.44 CFTR variants in the Scottish population

The table below shows the number of people with CF who carry at least one of each mutation. The groups are not mutually exclusive, as people with heterozygous mutations appear twice in the table.

These are the 20 most common mutations in the Scottish population. The full list of recorded mutations can be found in Appendix 2.

Nucleotide	Protein	Legacy name	N	%
c.1521_1523delCTT	p.Phe508del	F508del	880	90.8
c.1652G->A	p.Gly551Asp	G551D	92	9.5
c.350G->A	p.Arg117His	R117H	75	7.7
c.1624G->T	p.Gly542X	G542X	58	6.0
c.200C->T	p.Pro67Leu	P67L	55	5.7
c.3454G->C	p.Asp1152His	D1152H	24	2.5
c.1585-1G->A		1717-1G->A	20	2.1
c.1477C->T	p.Gln493X	Q493X	18	1.9
c.1679G->C	p.Arg560Thr	R560T	16	1.7
c.3909C->G	p.Asn1303Lys	N1303K	13	1.3
c.2657+5G->A		2789+5G->A	12	1.2
c.489+1G->T		621+1G->T	12	1.2
c.3873G->C	p.Gln1291His	Q1291H	11	1.1
c.3718-2477C->T		3849+10kbC->T	11	1.1
c.3528delC	p.Lys1177SerfsX15	3659delC	10	1.0
c.1558G->T	p.Val520Phe	V520F	8	0.8
c.178G->T	p.Glu60X	E60X	8	0.8
c.1210-12T[5]		5T	8	0.8
c.3140-26A->G		3272-26A->G	8	0.8
c.1364C->A	p.Ala455Glu	A455E	8	0.8

Sections 2 and 3: Centre-level analysis

Cystic fibrosis care in Scotland is led by seven regional centres, one stand-alone clinics. The breakdown of centres and clinics delivering paediatric and adult care is shown below:

	Paediatric	Adult	Total
Centres	4	3	7
Standalone clinics	1	0	1

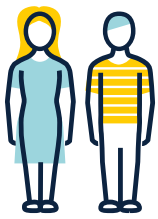
Section 2 shows analysis of data for individual CF centres. This allows people with CF, their families, and healthcare providers to review a centre's use of some medications and outcome data alongside national averages. This transparency is intended to help improve standards of care overall.

Lots of different factors can affect the outcomes of people with CF in centres, not all of which are within a centre's control. This might include the economic profile of the area, the age at which the person with CF was diagnosed and referred to the centre, and certain patient characteristics such as their gender, as well as facilities, care pathways, and the medical team providing care.

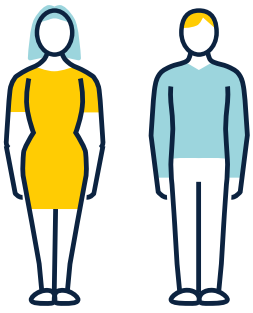
If a person with CF or a member of their family has questions about the results for their CF centre or clinic, they should discuss this with their CF team.

Full tables of the data are shown in Appendix 1.

Key to sections 2 & 3



Paediatric centre

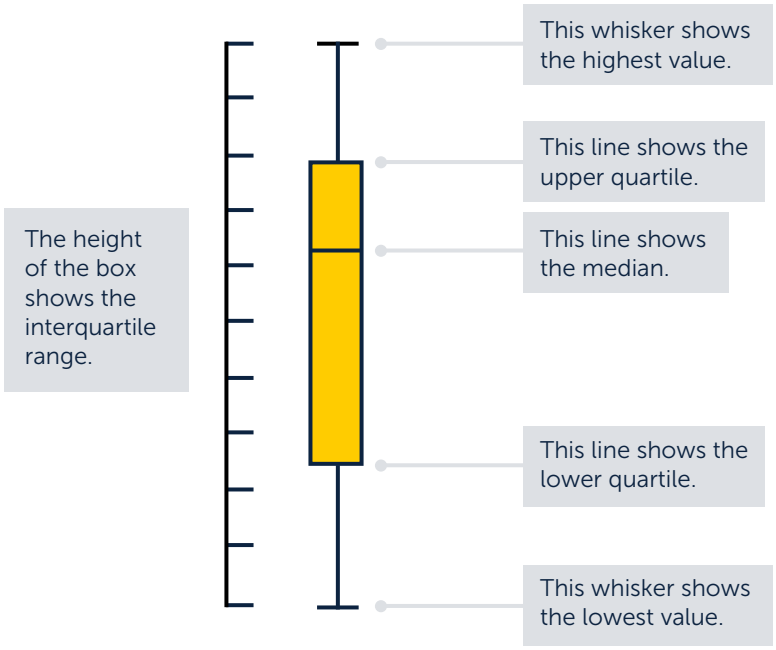


Adult centre

A Guide to the charts

Some of the data in this section are shown as 'box plots'.

Box plots



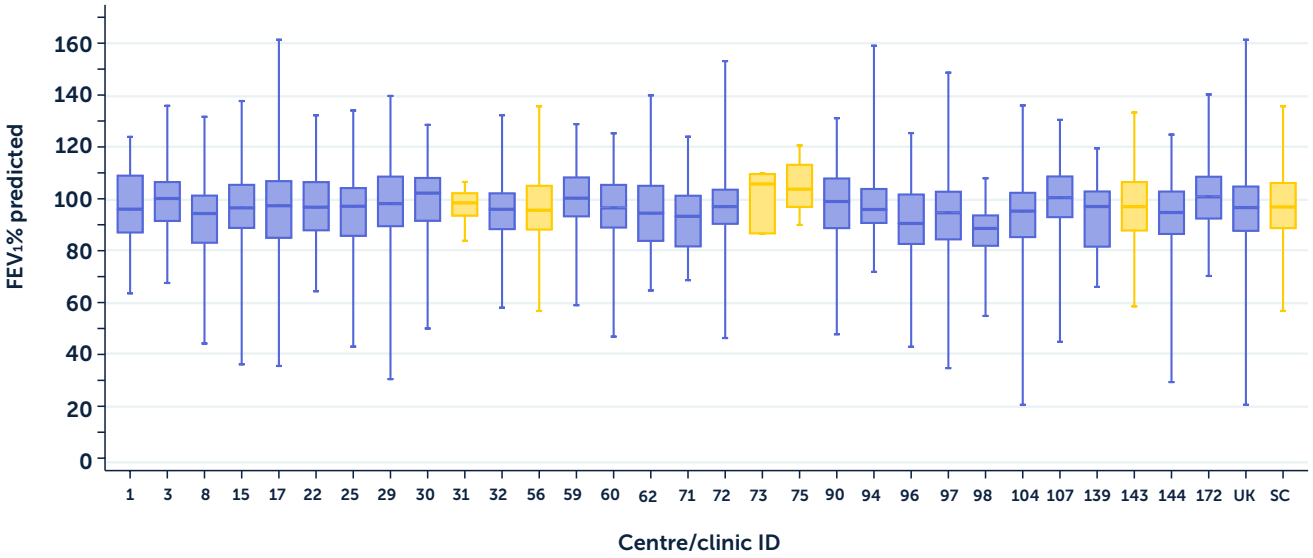
- The 'box' shows the middle half of the data for that centre, going from the first quartile to the third quartile. The longer the box, the more varied the data for that centre.
- The horizontal line within the box shows the median result for that centre.
- The 'whiskers' above and below the box show the highest and lowest values for that centre, excluding any outliers.
- The position of the box between the whiskers shows any skew in the data. If a box is towards the top of the whisker, more of the people for this centre were recorded at the high end of the scale.

Section 2: Paediatric centre analysis

Key

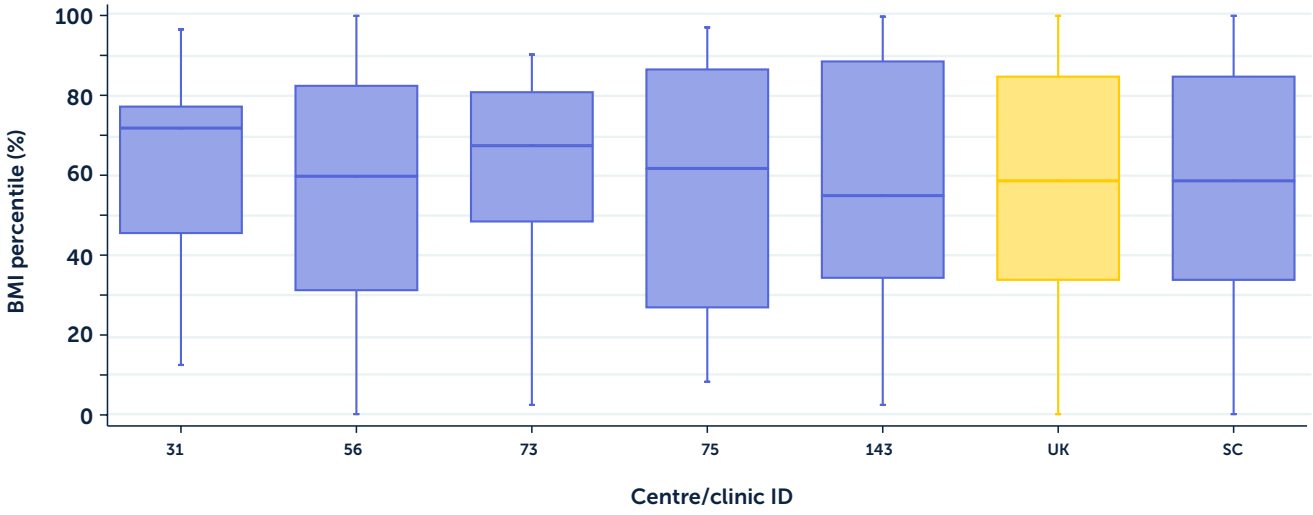
Services in the UK Services in Scotland

2.1 FEV_1 % predicted (GLI equations) among patients aged 6 and older without a history of lung transplant



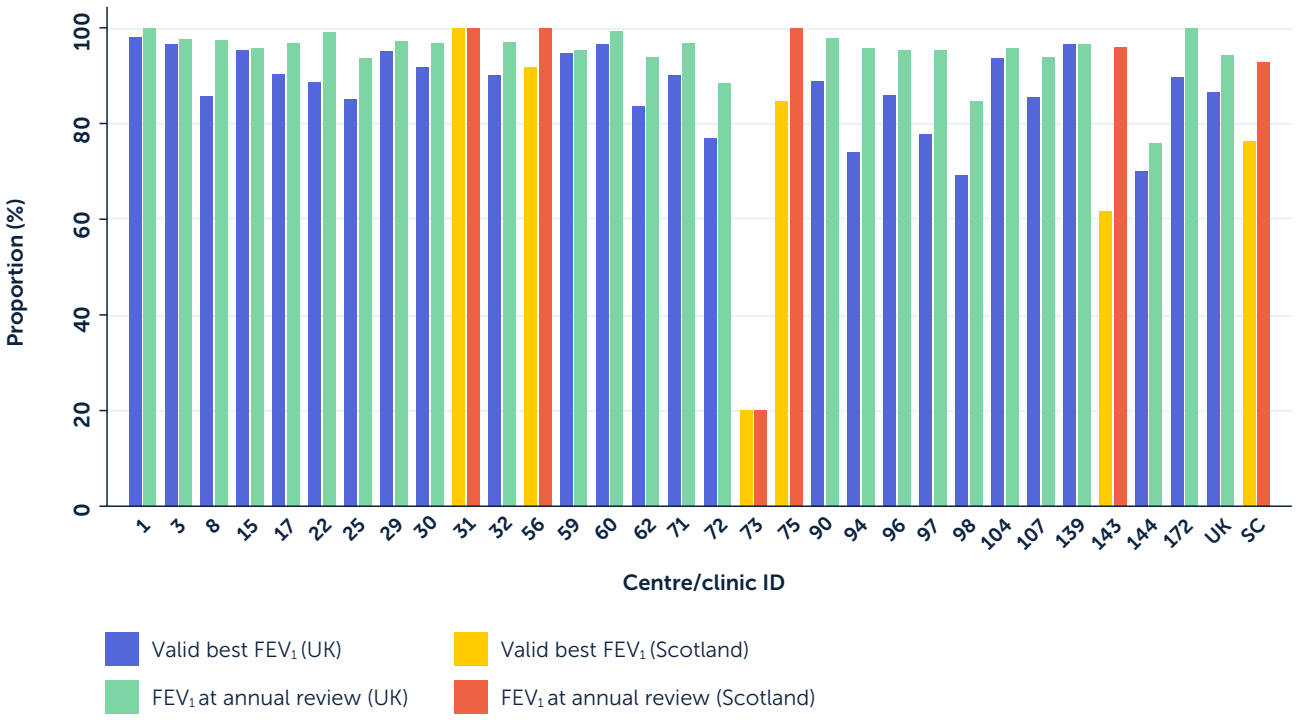
The mean FEV_1% predicted of patients attending paediatric centres/clinics in Scotland is 97.4% predicted (IQR: 88.5-106.3).

2.2 Body Mass Index (BMI) percentile among patients aged 2 – 15 years

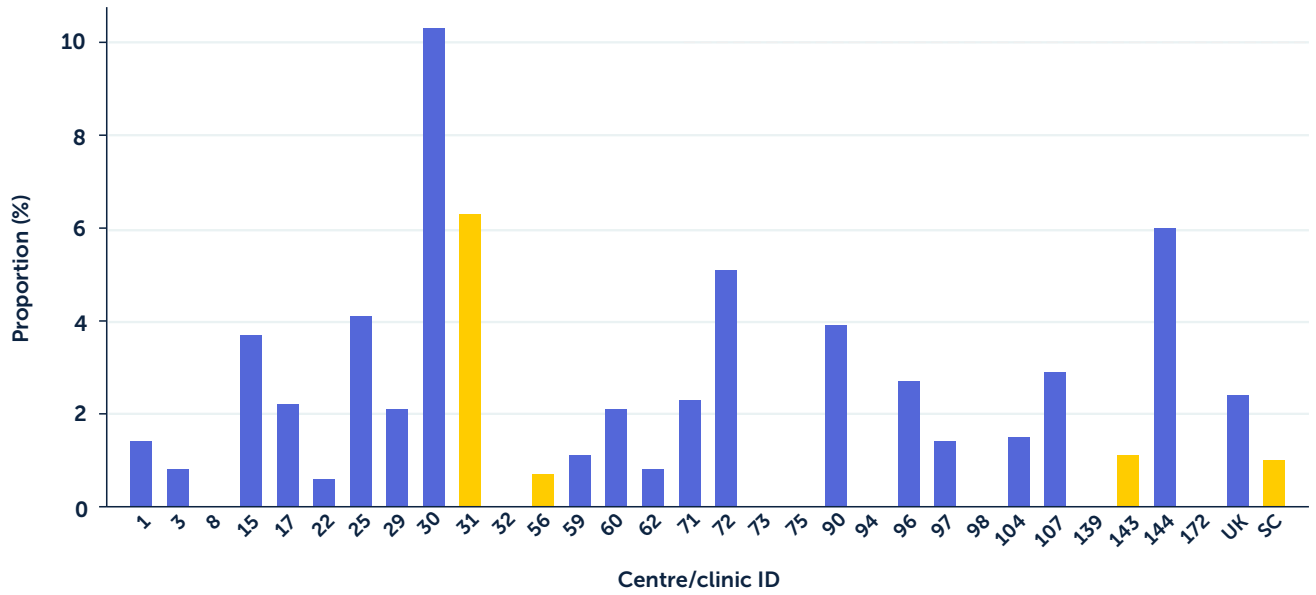


The median BMI percentile of patients attending paediatric centres/clinics in Scotland is 58.6 (IQR: 33.7 - 84.8).

2.3 Data completeness*

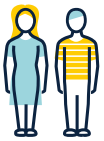


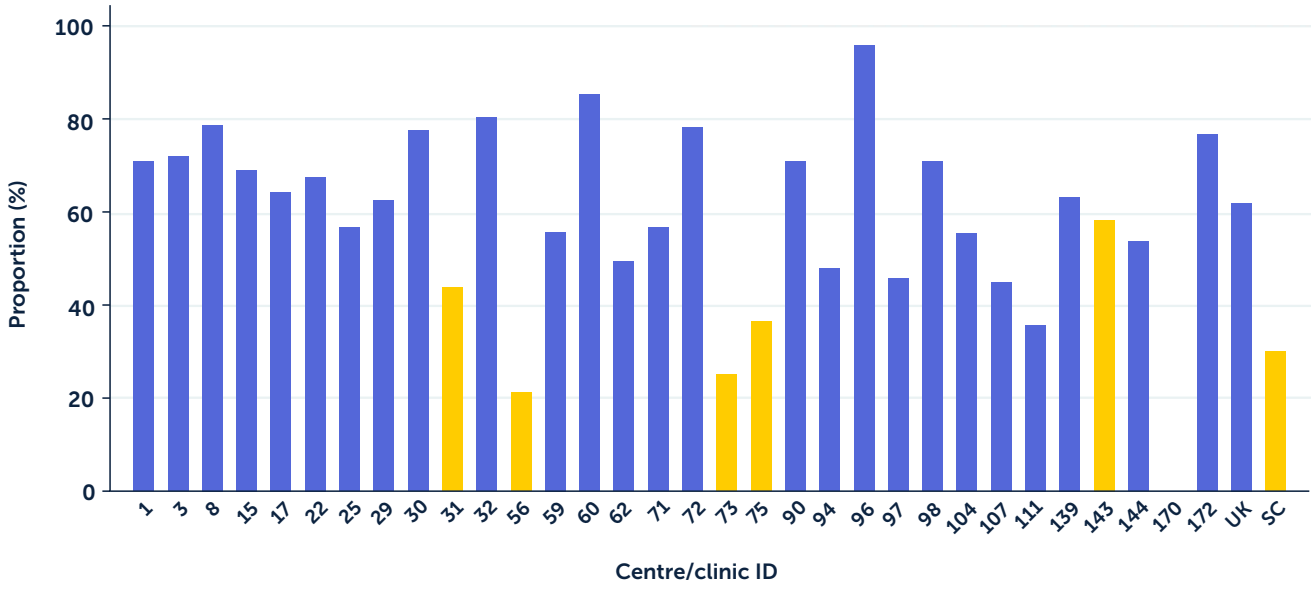
2.4 Proportion of patients with chronic *Pseudomonas aeruginosa*



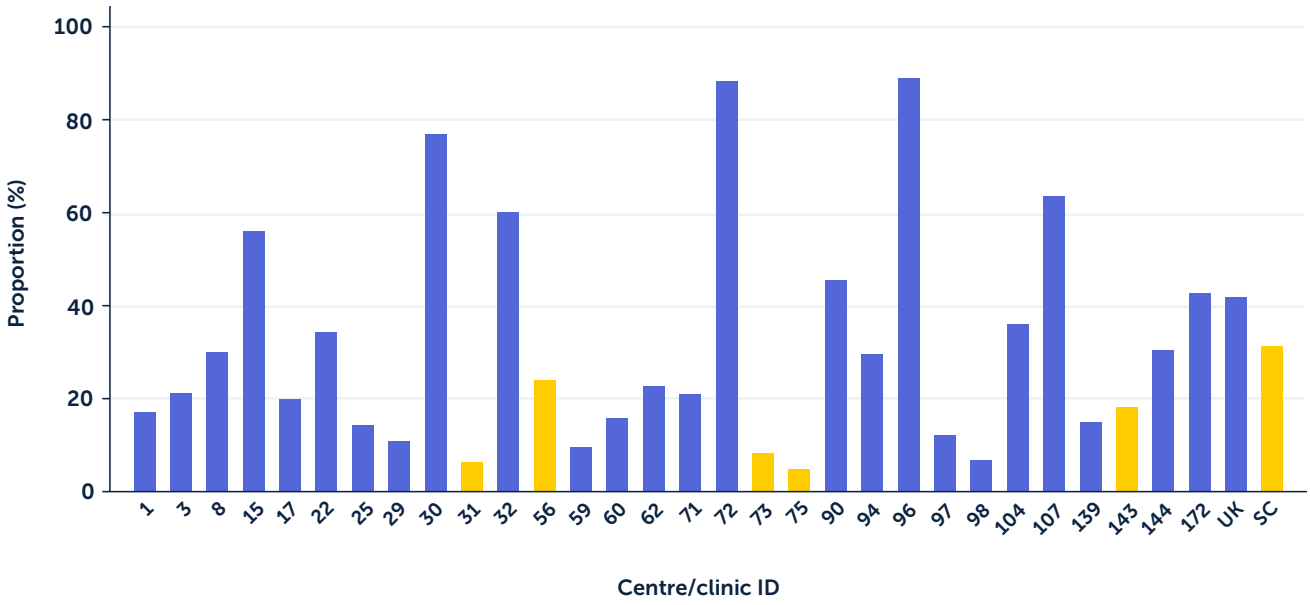
* The chart above shows the proportion of patients who had a valid best FEV_1% and an FEV_1% at annual review, excluding patients under six years of age. Best FEV_1% was considered valid if it was not missing, and the percent predicted was not more than 0.5% lower than the annual review value. For some patients there may be medical reasons why FEV_1 could not be taken, so centres may not be able to get 100% completeness.

2.5 Proportion of patients receiving DNase treatment





2.6 Proportion of patients receiving hypertonic saline treatment

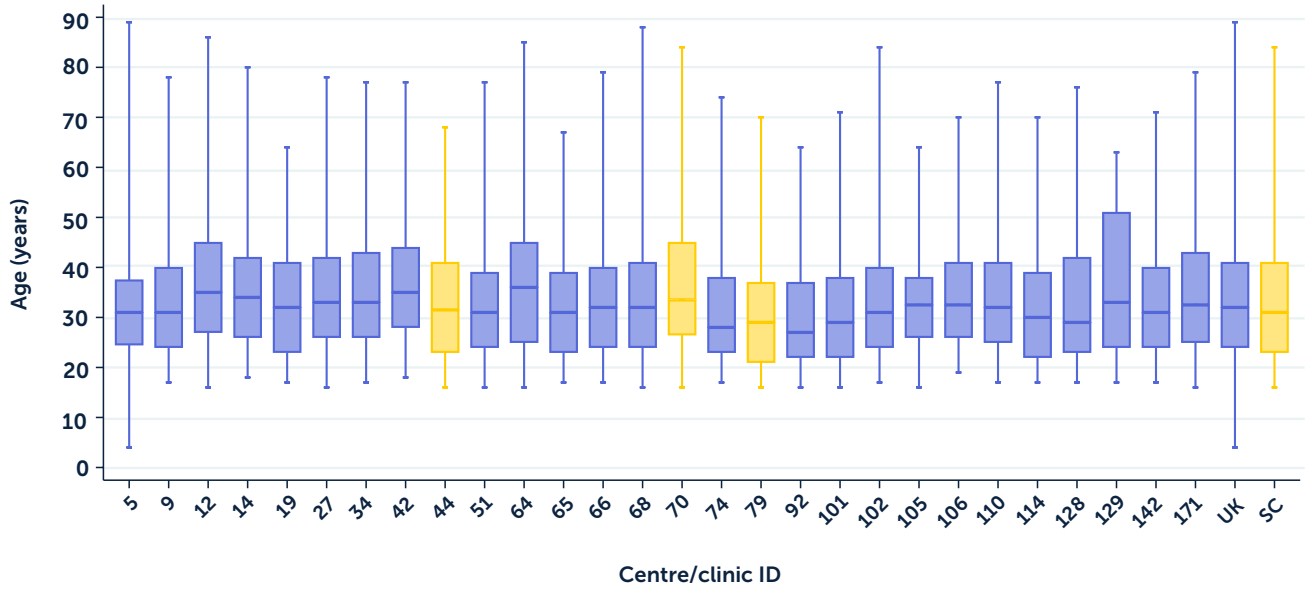


Section 3: Adult centre analysis

Key

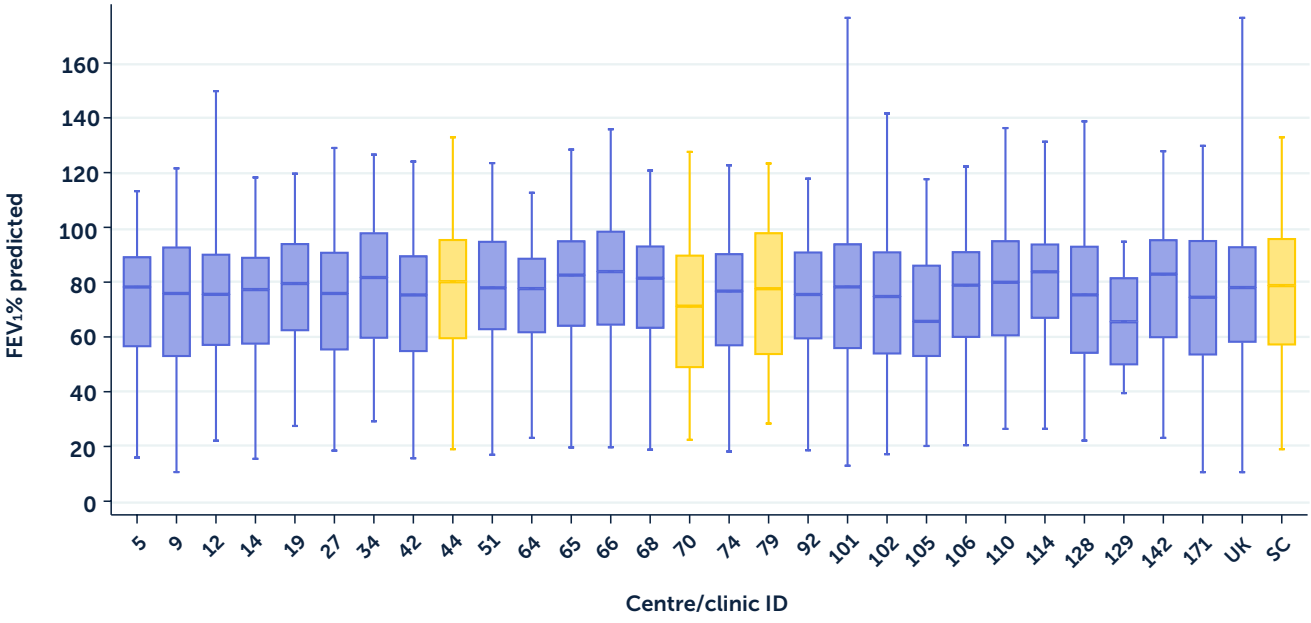
● Services in the UK ● Services in Scotland

3.1 Age distribution



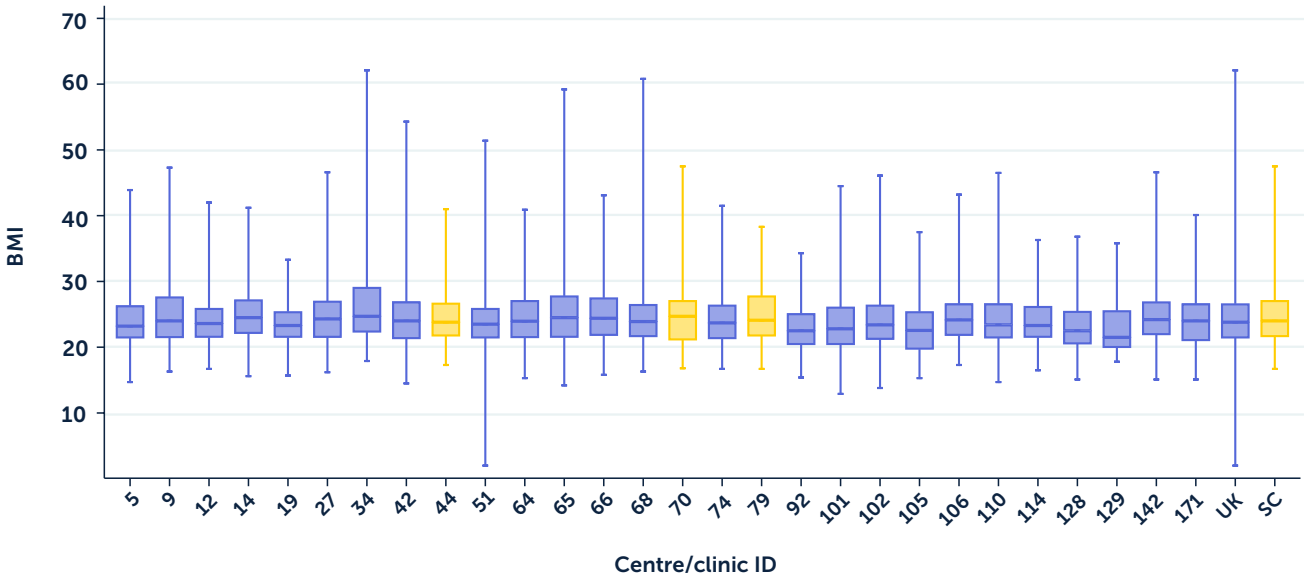
The median age of patients attending adult services in Scotland is 31 years (IQR: 23-41).

3.2 FEV_1 % predicted (GLI equations) at annual review, in patients aged 6 and over without a history of lung transplant



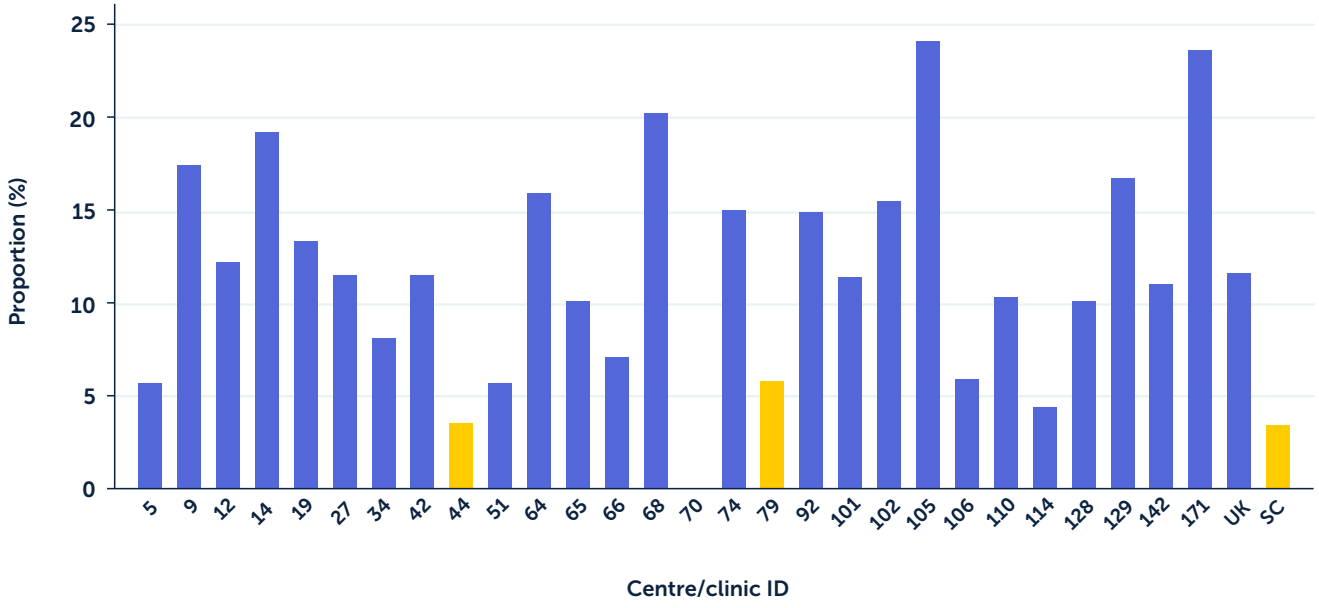
The median FEV_1% predicted of patients attending adult services in Scotland is 78.7% (IQR 57.0-96.0).

3.3 Body Mass Index (BMI) distribution among patients aged 16 years and older



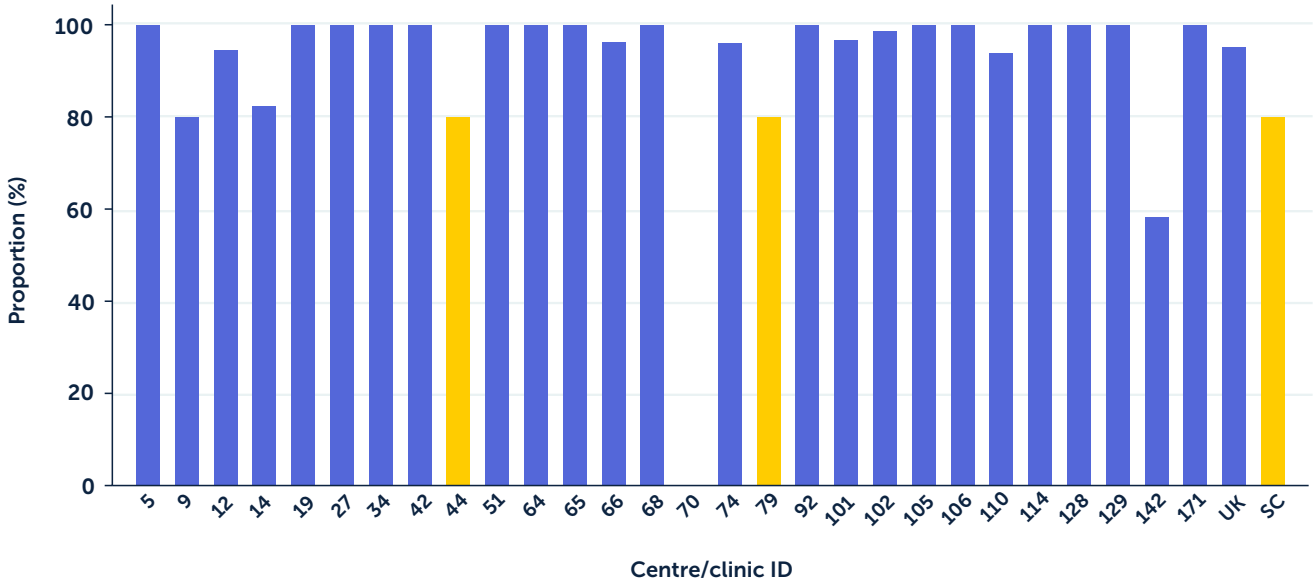
The median BMI of patients attending adult services in Scotland is 24.0 (IQR: 21.6 - 27.1).

3.4 Proportion of patients with chronic *Pseudomonas aeruginosa*



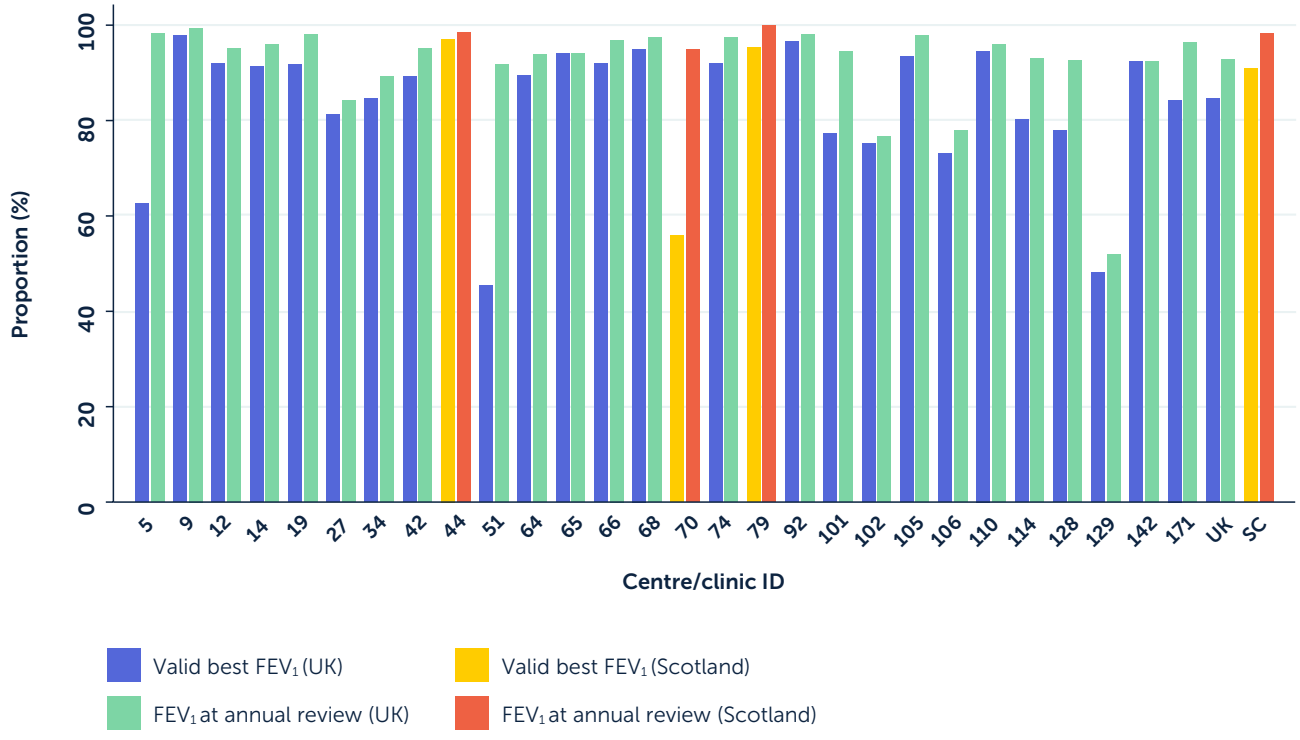
The proportion of patients attending adult services in Scotland with chronic *P. aeruginosa* is 3.2%.

3.5 Inhaled antibiotic use for patients with chronic *Pseudomonas aeruginosa*



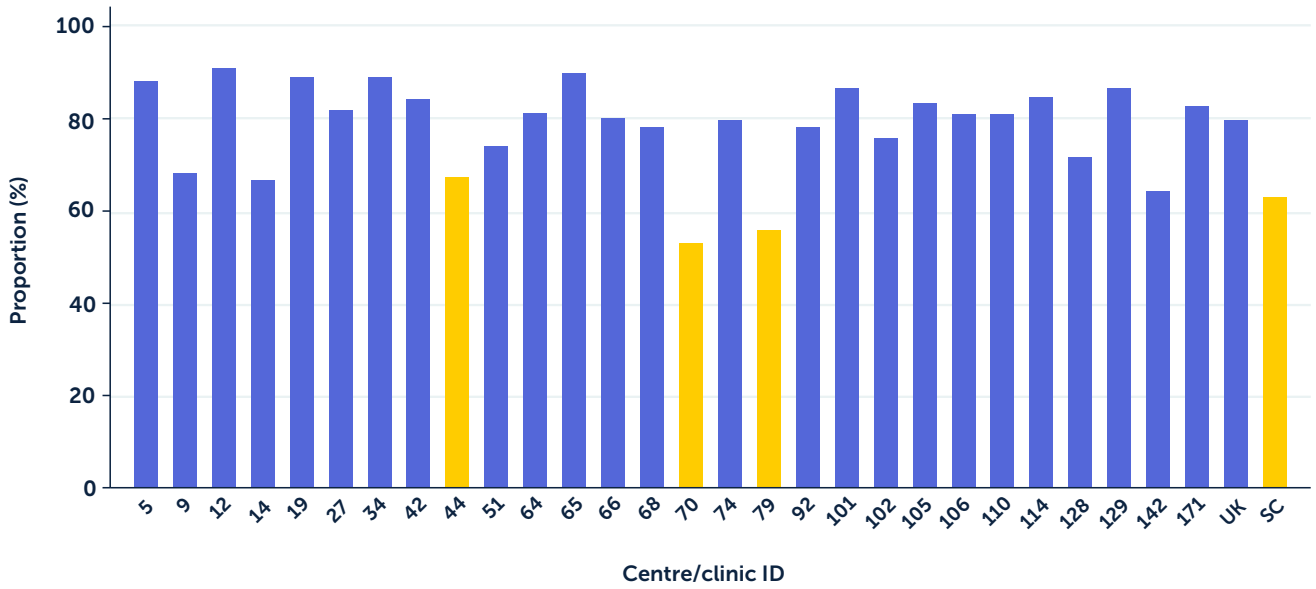
The proportion of chronic *P. aeruginosa* patients on inhaled antibiotics in Scotland is 80%.

3.6 Data completeness*



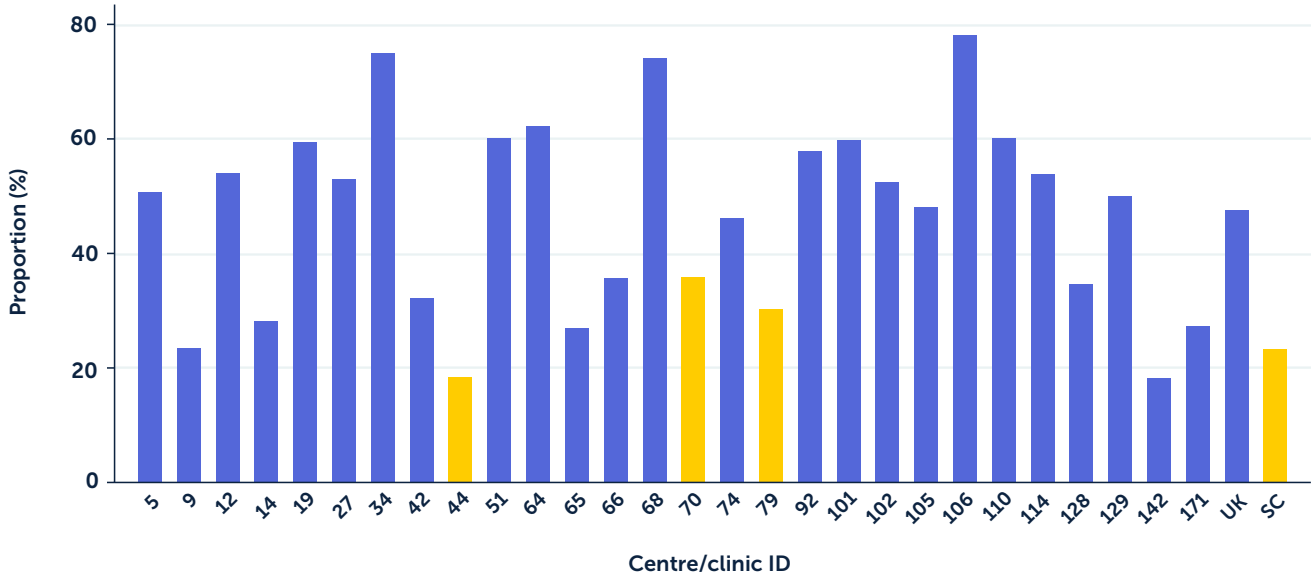
* FEV_1 was considered valid if it was not missing, and the percent predicted was not more than 0.5% lower than the annual review value. For some patients there may be medical reasons why FEV_1 could not be taken, so centres may not be able to get 100% completeness.

3.7 Proportion of patients receiving DNase treatment



The proportion of patients attending adult services in Scotland receiving DNase treatment is 63%.

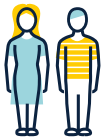
3.8 Proportion of patients receiving hypertonic saline or mannitol treatment



The proportion of patients attending adult services in Scotland receiving hypertonic saline or mannitol treatment is 23.3%.

Appendices and Glossary

Appendix 1: Centre-level data tables



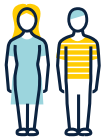
Paediatric centres/clinics providing data in 2024 – ordered alphabetically by location

Location	Name	Clinic ID	Total Active	Number with annual review
Scotland				
Aberdeen	Royal Aberdeen Children's Hospital	75	30	21
Dundee	Ninewells Hospital	73	25	24
Edinburgh	Royal Hospital for Sick Children	143	114	94
Glasgow	Royal Hospital for Sick Children	56	165	138
Inverness	Raigmore Hospital	31	16	16

Paediatric centres/clinics providing data in 2024 – ordered alphabetically by location

			BMI Percentile			
Location	Name	Clinic ID	Number*	Mean - unadjusted	Mean - adjusted	Median
Scotland						
Aberdeen	Royal Aberdeen Children's Hospital	75	19	58.4	58.2	61.7
Dundee	Ninewells Hospital	73	11	58.6	58.6	67.4
Edinburgh	Royal Hospital for Sick Children	143	89	58.9	58.9	56.3
Glasgow	Royal Hospital for Sick Children	56	128	56.6	56.6	59.8
Inverness	Raigmore Hospital	31	15	66.1	66.1	75.2

* Number with non-missing data.



	Age		FEV$_1$ % predicted at annual review				Best** FEV$_1$ % predicted			
Clinic ID	Mean	Median	Number*	Mean - unadjusted	Mean - adjusted	Median	Number*	Mean - unadjusted	Mean - adjusted	Median
75	7.8	7.0	13	105.1	104.1	103.7	13	110.0	108.7	106.9
73	8.4	9.1	<5	97.3	98.3	96.5	11	100.8	100.9	99.7
143	9.7	10.3	70	98.0	97.9	97.0	72	103.2	102.9	101.8
56	9.3	9.5	97	95.9	95.9	95.6	97	100.5	100.4	100.3
31	9.2	9.0	12	97.5	97.1	98.4	12	103.0	102.5	104.7

	Chronic pseudomonas		Having at least 1 IV days		Receiving DNase treatment		Receiving hypertonic saline or mannitol treatment		Inhaled antibiotic use among patients with chronic pseudomonas	
Clinic ID	Number*	Proportion (%)	Number*	Proportion (%)	Number*	Proportion (%)	Number*	Proportion (%)	Number*	Proportion (%)
75	0	0.0	<5	4.8	8	38.1	<5	14.3	0	0.0
73	0	0.0	0	0.0	5	20.8	<5	12.5	0	0.0
143	<5	1.1	12	12.8	53	56.4	16	17.0	<5	100.0
56	<5	0.7	19	13.8	24	17.4	47	34.1	<5	100.0
31	<5	6.2	<5	6.2	8	50.0	0	0.0	<5	100.0

* Redacted to adhere to statistical disclosure guidelines.

Adult centres/clinics providing data in 2024 – ordered alphabetically by location

Location	Name	Clinic ID	Total Active	Number with annual review
Scotland				
Aberdeen	Aberdeen Royal Infirmary	70	78	64
Edinburgh	Western General Hospital	44	298	288
Glasgow	Queen Elizabeth University Hospital	79	243	86

Adult centres/clinics providing data in 2024 – ordered alphabetically by location

				BMI Percentile		
Location	Name	Clinic ID	Number*	Mean - unadjusted	Mean - adjusted	Median
Scotland						
Aberdeen	Aberdeen Royal Infirmary	70	64	25.6	25.5	24.7
Edinburgh	Western General Hospital	44	287	24.6	24.7	23.8
Glasgow	Queen Elizabeth University Hospital	79	86	25.0	25.3	24.1

	Age		FEV$_1$ % predicted at annual review				Best** FEV$_1$ % predicted			
Clinic ID	Mean	Median	Number*	Mean - unadjusted	Mean - adjusted	Median	Number*	Mean - unadjusted	Mean - adjusted	Median
70	35.6	33.5	56	71.8	71.8	72.9	58	74.3	74.4	75.1
44	34.2	32.0	270	77.1	76.3	80.8	273	80.9	80.1	84.2
79	32.0	29.9	86	76.9	75.0	77.6	86	78.6	76.6	80.2

	Chronic pseudomonas		Having at least 1 IV days		Receiving DNase treatment		Receiving hypertonic saline or mannitol treatment		Inhaled antibiotic use among patients with chronic pseudomonas	
Clinic ID	Number*	Proportion (%)	Number*	Proportion (%)	Number*	Proportion (%)	Number*	Proportion (%)	Number*	Proportion (%)
70	0	0.0	<5	--**	13	20.3	<5	--**	0	0.0
44	10	5.0	32	11.1	131	45.5	30	10.4	7	70.0
79	5	6.0	18	20.9	19	22.1	10	11.6	<5	80.0

* Number with non-missing data.

** Where 'Best' values were missing, or lower than FEV$_1$% predicted taken at annual review, the annual review value was used.

Appendix 2: Full list of CFTR variants in the Scottish population

The table below shows the number of people with CF who carry at least one of each mutation.

The groups are not mutually exclusive, as people with heterozygous mutations appear twice in the table.

Nucleotide	Protein	Legacy name	N	%
c.1521_1523delCTT	p.Phe508del	F508del	880	90.8
c.1652G->A	p.Gly551Asp	G551D	92	9.5
c.350G->A	p.Arg117His	R117H	75	7.7
c.1624G->T	p.Gly542X	G542X	58	6.0
c.200C->T	p.Pro67Leu	P67L	55	5.7
c.3454G->C	p.Asp1152His	D1152H	24	2.5
c.1585-1G->A		1717-1G->A	20	2.1
c.1477C->T	p.Gln493X	Q493X	18	1.9
c.1679G->C	p.Arg560Thr	R560T	16	1.7
c.3909C->G	p.Asn1303Lys	N1303K	13	1.3
		Missing/not known	12	1.2
c.2657+5G->A		2789+5G->A	12	1.2
c.489+1G->T		621+1G->T	12	1.2
c.3873G->C	p.Gln1291His	Q1291H	11	1.1
c.3718-2477C->T		3849+10kbC->T	11	1.1
c.3528delC	p.Lys1177SerfsX15	3659delC	10	1.0
c.1558G->T	p.Val520Phe	V520F	8	0.8
c.178G->T	p.Glu60X	E60X	8	0.8
c.1210-12T[5]		5T	8	0.8
c.3140-26A->G		3272-26A->G	8	0.8
c.1364C->A	p.Ala455Glu	A455E	8	0.8
c.2657+2_2657+3insA		2789+2insA	7	0.7
c.3846G->A	p.Trp1282X	W1282X	6	0.6
c.948delT	p.Phe316LeufsX12	1078delT	5	0.5
c.1705T->G	p.Tyr569Asp	Y569D	5	0.5
c.1766+1G->A		1898+1G->A	5	0.5
c.1721C->A	p.Pro574His	P574H	<5	-
c.1329_1330insAGAT	p.Ile444ArgfsX3	1461ins4	<5	-
c.1519_1521delATC	p.Ile507del	I507del	<5	-
c.1657C->T	p.Arg553X	R553X	<5	-
c.3468G->A		3600G->A	<5	-
c.254G->A	p.Gly85Glu	G85E	<5	-
c.54-5940_273+10250del21kb	p.Ser18ArgfsX16	CFTRdele2,3	<5	-
c.2012delT	p.Leu671X	2143delT	<5	-
c.579+3A->G		711+3A->G	<5	-
c.2052delA	p.Lys684AsnfsX38	2184delA	<5	-
c.429delT	p.Phe143LeufsX10	557delT	<5	-
c.2988+1G->A		3120+1G->A	<5	-
c.617T->G	p.Leu206Trp	L206W	<5	-
c.1680A->C	p.Arg560Ser	R560S	<5	-
c.3196C->T	p.Arg1066Cys	R1066C	<5	-
c.223C->T	p.Arg75X	R75X	<5	-
c.164+2T->C		296+2T->C	<5	-
c.3884_3885insT	p.Ser1297PhefsX5	4016insT	<5	-
c.2490+1G->A		2622+1G->A	<5	-
c.653T>A	p.Leu218X	L218X	<5	-

Nucleotide	Protein	Legacy name	N	%
c.1367T->C	p.Val456Ala	V456A	<5	-
c.3737C->T	p.Thr1246Ile	T1246I	<5	-
c.[1210-12[5];1210-34TG[12]]		5T;TG12	<5	-
c.461dup	p.Ala155SerfsX4	593insT	<5	-
c.1006_1007insG	p.Ile336SerfsX28	1138insG	<5	-
c.2125C->T	p.Arg709X	R709X	<5	-
c.3705T->G	p.Ser1235Arg	S1235R	<5	-
c.[1210-12[5];1210-34TG[13]]		5T;TG13	<5	-
c.349C->T	p.Arg117Cys	R117C	<5	-
c.3353C->T	p.Ser1118Phe	S1118F	<5	-
c.2900T->C	p.Leu967Ser	L967S	<5	-
c.2583delT	p.Phe861LeufsX3	2711delT	<5	-
c.273+1G->A		405+1G->A	<5	-
c.[1521_1523delCTT;3080T->C]	p.[Phe508del;Ile1027Thr]	F508del;I1027T	<5	-
c.1645A->C or c.1647T->G or c.1647T->A	p.Ser549Arg	S549R	<5	-
c.292C->T	p.Gln98X	Q98X	<5	-
c.(3873+1_3874-1)_(3963+1_3964-1)		CFTRdele21	<5	-
del c.933C>G or c.933C>A	p.Phe311Leu	F311L	<5	-
c.743+1G>C	p.?	875+1G->C	<5	-
c.274G->A	p.Glu92Lys	E92K	<5	-
c.1209+1G->A		1341+1G->A	<5	-
c.1753G->T	p.Glu585X	E585X	<5	-
c.509G->A	p.Arg170His	R170H	<5	-
c.2859_2890delACATTCTGTTCTT CAAGCACCTATGTCAACCC	p.Leu953PhefsX11	2991del32	<5	-
c.1466C->A	p.Ser489X	S489X	<5	-
c.2988G->A		3120G->A	<5	-
c.1029delC	p.Cys343X	1161delC	<5	-
c.233dupT	p.Trp79LeufsX32	365-366insT	<5	-
c.443T->C	p.Ile148Thr	I148T	<5	-
c.1055G->A	p.Arg352Gln	R352Q	<5	-
c.1327G->T	p.Asp443Tyr	D443Y	<5	-
c.(53+1_54-1)_(489+1_490-1)del		CFTRdele2-4	<5	-
c.3475T->C	p.Ser1159Pro	S1159P	<5	-
c.4147_4148insA	p.Ile1383AsnfsX3	4279insA	<5	-
c.262_263delTT	p.Leu88IlefsX22	394delTT	<5	-
c.1986_1989delAACT	p.Thr663ArgfsX8	2118del4	<5	-
c.3276C->A or c.3276C->G	p.Tyr1092X	Y1092X	<5	-
c.2051_2052delAAinsG	p.Lys684SerfsX38	2183AA->G or 2183delAA->G	<5	-
c.1040G->C	p.Arg347Pro	R347P	<5	-
c.1523T->G	p.Phe508Cys	F508C	<5	-
c.3484C->T	p.Arg1162X	R1162X	<5	-
c.3158C->T	p.Thr1053Ile	T1053I	<5	-
c.1000C->T	p.Arg334Trp	R334W	<5	-
c.1538A->G	p.Asp513Gly	D513G	<5	-
c.2855T->C	p.Met952Thr	M952T	<5	-
c.3208C->T	p.Arg1070Trp	R1070W	<5	-
c.1022_1023insTC	p.Phe342HisfsX28	1154insTC	<5	-
c.3476C->T	p.Ser1159Phe	S1159F	<5	-
c.3197G->A	p.Arg1066His	R1066H	<5	-
c.3080T->C	p.Ile1027Thr	I1027T	<5	-
'Other' selected			16	1.7

Appendix 3: Legacy names lists for modulator eligibility*

FDA list of CFTR variants potentially responsive to elexacaftor/tezacaftor/ivacaftor[1]

3141del9	E92K	G628R	L320V	R170H	S549R
546insCTA	F1016S	G85E	L346P	R258G	S589N
A1006E	F1052V	G970D	L453S	R31L	S737F
A1067T	F1074L	H1054D	L967S	R334L	S912L
A120T	F1099L	H1085P	L997F	R334Q	S945L
A234D	F191V	H1085R	M1101K	R347H	S977F
A349V	F311del	H1375P	M152V	R347L	T1036N
A455E	F311L	H139R	M265R	R347P	T1053I
A46D	F508C	H199Y	M952I	R352Q	T338I
A554E	F508C;S1251N†	H939R	M952T	R352W	V1153E
D110E	F575Y	I1027T	P205S	R553Q	V1240G
D110H	G1061R	I1139V	P574H	R668C	V1293G
D1152H	G1069R	I1269N	P5L	R74Q	V201M
D1270N	G1244E	I1366N	P67L	R74W	V232D
D192G	G1249R	I148T	Q1291R	R74W;D1270N†	V456A
D443Y	G126D	I175V	Q237E	R74W;V201M;D1270N†	V456F
D443Y;G576A;R668C†	G1349D	I336K	Q237H	R74W;V201M†	V562I
D579G	G178E	I502T	Q359R	R751L	V754M
D614G	G178R	I601F	Q98R	R75Q	W1098C
D836Y	G194R	I618T	R1066H	R792G	W1282R
D924N	G194V	I807M	R1070Q	R933G	W361R
D979V	G27R	I980K	R1070W	S1159F	Y1014C
E116K	G314E	K1060T	R1162L	S1159P	Y1032C
E193K	G463V	L1077P	R117C	S1251N	Y109N
E403D	G480C	L1324P	R117G	S1255P	Y161D
E474K	G551D	L1335P	R117H	S13F	Y161S
E56K	G551S	L1480P	R117L	S341P	Y563N
E588V	G576A	L15P	R117P	S364P	
E60K	G576A;R668C†	L165S	R1283M	S492F	
E822K	G622D	L206W	R1283S	S549N	

* see 1.35a and 1.35b which reference these lists

1 **https://pi.vrtx.com/files/uspi_elexacaftor_tezacaftor_ivacaftor.pdf** (accessed prior to December 2024, has since been updated to include 94 additional mutations as of December 2024, current prescribing information contains the additional 94 mutations listed here: **https://www.cff.org/news/2024-12/fda-approves-trikafta-additional-rare-cftr-mutations**. These were not considered in this report as they were not named eligible variants during the 2024 annual review year.

FDA list of additional[2] CFTR variants potentially responsive to either tezacaftor/ivacaftor or ivacaftor

	Named variant
Tezacaftor/ivacaftor and Ivacaftor extension list eligible	2789+5G->A
	3272-26A->G
	3849+10kbC->T
	711+3A->G
	D443Y;G576A;R668C
	E831X
	F508C;S1251N
Tezacaftor/ivacaftor ONLY extension list eligible	G576A;R668C
	R74W;D1270N
	R74W;V201M
	R74W;V201M;D1270N

Potentially responsive CFTR variants according to French Compassionate Use Programme[3]

	Named variant
Franch compassionate use programme	2789+5G->A
	3041-15T->G
	3272-26A->G
	3849+10kbC->T
	N1303K
	R1066C
	R334W

2 **https://www.cff.org/managing-cf/cftr-modulator-therapies**; there are a total of 154 named variants potentially responsive to tezacaftor/ivacaftor and 97 variants potentially responsive to ivacaftor. The table above is the list of variants responsive to either tezacaftor/ivacaftor or ivacaftor which are not named as potentially responsive to elexacaftor/tezacaftor/ivacaftor.

3 Burgel PR et al. Eur Respir J. Feb 2023; variants may overlap with named variants in list 4.2.

Glossary

Word/Phrase	Meaning
2024	1 January 2024–31 December 2024.
ABPA (allergic bronchopulmonary aspergillosis)	When a person develops a respiratory allergic reaction to *Aspergillus fumigatus*.
Arthritis	A condition causing pain and inflammation in the joints.
Arthropathy	A condition causing pain in the joints.
Asthma	A respiratory condition causing reversible episodes of difficulty breathing, often associated with wheezing.
***Burkholderia cepacia* complex**	*B. cepacia* complex is a group of bacteria, some of which threaten the health of people with cystic fibrosis.
BMI (Body Mass Index)	A measure designed to show whether a person is a healthy weight for their height.
CF	Cystic fibrosis.
CFTR (cystic fibrosis transmembrane conductance regulator)	A protein at the cell surface that controls the salt and water balance across a cell. The gene that causes cystic fibrosis is the blueprint for the CFTR protein. Everyone has two copies of the gene for CFTR. To be born with cystic fibrosis, both CFTR genes must be affected by a CF-causing mutation.
Chronic	Persistent, or long-lasting.
Cirrhosis	A chronic liver disease.
CI (confidence interval)	A way of expressing how certain we are about our statistical estimates of a clinical measure (eg BMI). It gives a range of results that is likely to include the 'true' value for the population. A narrow confidence interval indicates a more precise estimate. A wide confidence interval indicates more uncertainty about the true value of the clinical measure - often because a small group of patients has been studied. The confidence interval is usually stated as '95% CI', which means that the range of values has a 95 in 100 chance of including the 'true' value.
Enzymes	Biological molecules that help complex reactions, such as the digestion of food, occur in the body.
FEV_1 (forced expiratory volume in one second)	This is the amount of air that a person can blow out of the lungs in the first second of a forced exhaled breath. People with healthy lungs can blow out most of the air held in this time.
FEV_1% predicted	The FEV_1 can be converted from absolute litres of air blown out into a predicted percentage (%). A healthy range for % predicted is calculated from a very large population sample, and is normally considered to be between 80-120% predicted.
Fibrosing colonopathy	A condition causing narrowing of part of the colon.
Gall bladder	The small sac-shaped organ under the liver that stores bile after it is secreted by the liver, before it is released into the intestine.
Gastrointestinal (GI) tract	The GI tract is an organ system responsible for digesting food, absorbing nutrients and expelling waste.
Genotype	Part of the genetic makeup of a cell, organism or individual that usually controls a particular characteristic (known as a phenotype).
GORD (gastro-oesophageal reflux disease)	A chronic symptom of damage caused by stomach acid coming up from the stomach into the oesophagus.
GI bleed	Bleeding in the gastrointestinal tract.
GLI equations	Global Lung Initiative, the equation used for calculating FEV_1% predicted from absolute FEV_1, which takes into account age, gender, height and ethnicity.
H. influenza	*Haemophilus influenza* is a bacterium that can cause serious illness.
Haemoptysis	The coughing up of blood.
Hepatobiliary disease	A liver or biliary disorder.
Heterozygous	Everyone living with cystic fibrosis has two mutations of the gene for CFTR, one inherited from their mother and one from their father. Someone who has two different mutations is heterozygous.

Word/Phrase	Meaning
Homozygous	Everyone living with cystic fibrosis has two mutations of the gene for CFTR, one inherited from their mother and one from their father. If both mutations (or genotypes) are the same, the person is said to be homozygous.
Hypertension	High blood pressure.
Incidence	The number of people newly diagnosed with a condition in the given year.
IQR (interquartile range)	Also called the mid-spread, or middle fifty, IQR is a measure of the spread of data. It shows the difference between the upper and lower quartiles. IQR = Q3 – Q1.
Mean	A type of average, calculated by adding up all the values and dividing by the number of values.
Median	The middle number, when all numbers are arranged from smallest to largest.
Median age of death	Median age of death is based on the people with CF who died in any given year.
Median predicted survival age	A prediction of how long we expect half of the people with CF born today live for.
MRSA	Methicillin-resistant *Staphylococcus aureus* is a type of bacteria that is resistant to a number of widely used antibiotics.
Mutation	A mutation is a change in a gene. When both of a child's parents are carriers of a CF-causing mutation there is a 25% chance that the child will have cystic fibrosis. There are over 1,400 different mutations of the CFTR gene that can cause cystic fibrosis.
Nasal polyps	Small, sac-like growths of inflamed mucus membrane caused by chronic inflammation of the nasal lining.
NBS (newborn screening)	Newborn screening is part of the heel prick blood spot testing carried out on all babies at 5-7 days of age. The blood sample is tested for a number of conditions, including cystic fibrosis.
NTM (non-tuberculous mycobacteria)	A mycobacterium that does not cause tuberculosis, but which can cause respiratory infection. There are several known types.
Osteopenia	A medical condition less severe than osteoporosis, where the mineral content of bone is reduced.
Osteoporosis	A condition where the bones become brittle from loss of tissue.
Pancreas	An organ in the digestive system that produces insulin and digestive enzymes.
Pancreatitis	Inflammation of the pancreas.
Peptic ulcer	An open sore that develops in the lining of the stomach, also known as a stomach ulcer.
Percentile	A percentile shows where a value stands, relative to the rest of the data. If a value is higher than 90% of the rest of the data, it is at the 90th percentile.
Pneumothorax	A collection of air in the cavity between the lungs and the chest wall causing collapse of the lung on the affected side.
Portal hypertension	High blood pressure in the portal vein system, which is the blood system of the liver.
Prenatal	Before birth, while the baby is still in the womb.
Prevalence	The overall number of people with the condition in the last 12 months.
Pseudomonas aeruginosa	A tough bacterial strain. Rarely affecting healthy people, it can cause a wide range of infections, particularly in those with a weakened immune system.
Rectal prolapse	When the rectal wall slides through the anus.
Renal	Relating to the kidneys.
Staphylococcus aureus	*S. aureus* is a bacterium that can cause disease if it enters the body.
Sinus disease	When the sinuses, which are usually filled with air, are full of thick sticky mucus.
Statistically significant	This phrase means that after careful calculations there is a definite difference between two groups, which is not simply a result of chance.

Cystic Fibrosis Trust

Cystic Fibrosis Trust is the charity uniting people to stop cystic fibrosis. Our community will improve care, speak out, support each other and fund vital research as we race towards effective treatments for all.

We won't stop until everyone can live without the limits of cystic fibrosis.

cysticfibrosis.org.uk

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