

UK Cystic Fibrosis Registry **Annual Data Report 2023 –** **Scotland**

March 2025

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UK Cystic Fibrosis Registry Annual Data Report 2023 – Scotland

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Contact information

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Executive summary



Welcome to the 2023 UK CF Registry Annual Data Report for Scotland. I hope you find things that give cause for celebration and pause for thought on what might be done to make care better for people with CF moving forward.

There's a lot of data presented here – so some things that I've reflected on are summarised here:

The age distribution (Section 1.3) is shifting as we would expect – though within Scotland this is quite variable, particularly around adolescence with a larger proportion transitioning to adult care now than 10 years previously.

There has been an increase in the proportion of 10 to 14-year-olds in the overweight BMI category with nearly 1 in 4 above the 90th percentile for BMI. (Section 1.7)

For adults, the change observed has been even more stark over the past five years, with an increase in the proportion of people in the overweight and obese BMI categories. Going forward, proportionate and personalised advice on the role of integrated exercise and tailored dietary input for a generation predominantly exposed to modulator therapies, should gain greater clinical focus if people with cystic fibrosis are to lead healthy, longer lives.

Lung health, as measured by FEV₁, is making transformational leaps (Section 1.15). These are to be heralded. However, these figures include data for those who have no access to modulator therapies, for whom there is yet to be medications offering this same transformation. For those with access to modulator therapies, there needs to be a continual refresher that this transformation is reliant on, consistent adherence.

Frequent airway sampling continues to be a mainstay in paediatric care (94% had three samples or more) but has halved in adult care over the past five years (72% down to 35%). Drier airways (and less sputum for samples) are of course a good thing, but these data highlight the need to develop better methods of identifying and (re)defining chronic lower airway infection.

With a population thankfully living longer, we need Registry data to help inform and resolve any potential long-term impacts of early life exposures, such as high fat diets, gut inflammation, ototoxic medicines and burden of treatment on our community. Section 1.21 begins to build this picture – and clinical engagement with this data will be pivotal in identifying early signs of those longer-term impacts.

Mucoactive therapies do appear to be reducing in use, particularly for hypertonic saline in those over 16 years of age, where use has halved over the last five years. DNase use is however declining at a much slower pace as people evaluate their individual response to modulator therapies and the risk/benefit of stopping. Results from the Registry-based trial CFSTORM will help inform future care in this space.

CFTR modulator use is now standard of care for most people with CF (Section 1.35), however, there remains individuals with eligible genotypes who have no record of CFTR modulator use. Patients, parents and clinic staff will all wish to ensure that eligibility is reviewed in those not currently taking CFTR modulators, to ensure any prior assessment of eligibility remains correct.

Numbers are small, but the reduction in patients evaluated and receiving lung and liver transplantation over the past five years, paired with an increase in life expectancy, is really positive to see.

The proportion of people living with CF in Scotland with annual review data was 76% in 2023 - our learning is based on just over three-quarters of the population. Entering data into a data registry can be tough and time consuming, but the data summarised in this report will help clinicians improve care of their patients so that people with CF live better lives now and in the future. From all in the UK CF Registry team – thank you.

With very best wishes

Steve Cunningham

Professor of Paediatric Respiratory Medicine
University of Edinburgh

Introduction

This report is aimed at anyone who is interested in the health, care, and outcomes of people with cystic fibrosis (CF) in the UK. 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 60.

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:



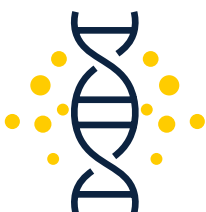
helping people with CF and their families understand CF, and make informed decisions



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 their patients' disease severity



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 1 of the UK Cystic Fibrosis Registry 2023 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

| | 2023 | |
|---|-------------|----------|
| | UK | Scotland |
| CF patients registered ¹ | 11318 | 972 |
| Excluding diagnoses that year | 11146 | 956 |
| CF patients with an annual review; n ² | 10344 | 723 |
| Age in years; median ³ | 22 | 22 |
| All newly diagnosed patients (newborn screening and other) ⁴ | 173 | 16 |
| Number of patients born identified by newborn screening ⁴ | 124 | 9 |
| Age at diagnosis in months; median ³ | 1.3 | 1.3 |
| Adults aged 16 years and over; % ³ | 63.7 | 45.9 |
| Males; % ³ | 53.3 | 53.9 |
| Genotyped; % ³ | 99.4 | 99.7 |
| Total deaths reported during annual review year (%) ⁵ | 4.9 | 6.0 |
| Age at death in years; median (95% CI) ⁵ | 46 (37, 55) | 55* |



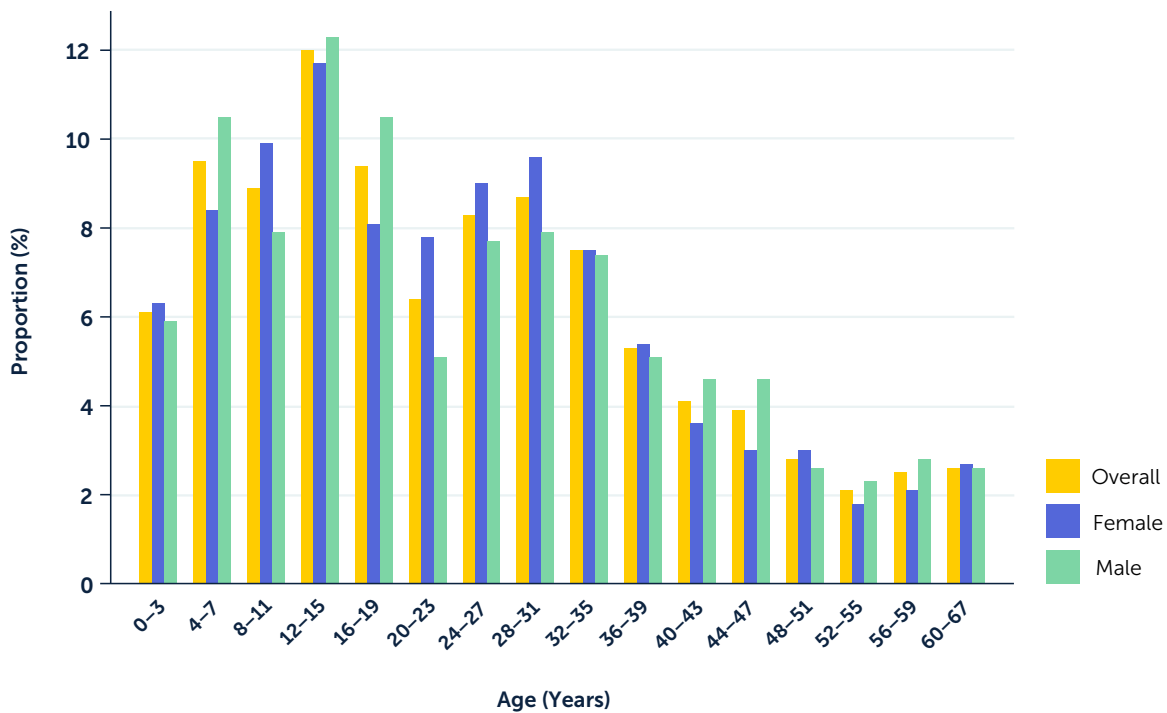
Annual review: A Registry annual review form contains a combination of data relating to a person with CF's yearly annual review appointment at their CF centre, and their clinical care and health over the past 12 months.

Notes:

- 1 Number of patients diagnosed with CF, seen in the past two years, and alive at 1 January in the given year.
 - 2 As patients newly diagnosed in a given year may not have their first annual review in the same year, the proportion with an annual review is calculated from the total registered excluding those diagnosed in the given year.
 - 3 Calculated from patients with an annual review in the given year (see footnote 2 above).
 - 4 Calculated from all patients registered on the database.
 - 5 Calculated from all registered patients who died in the given year.
- * CI not provided due to small sample size.

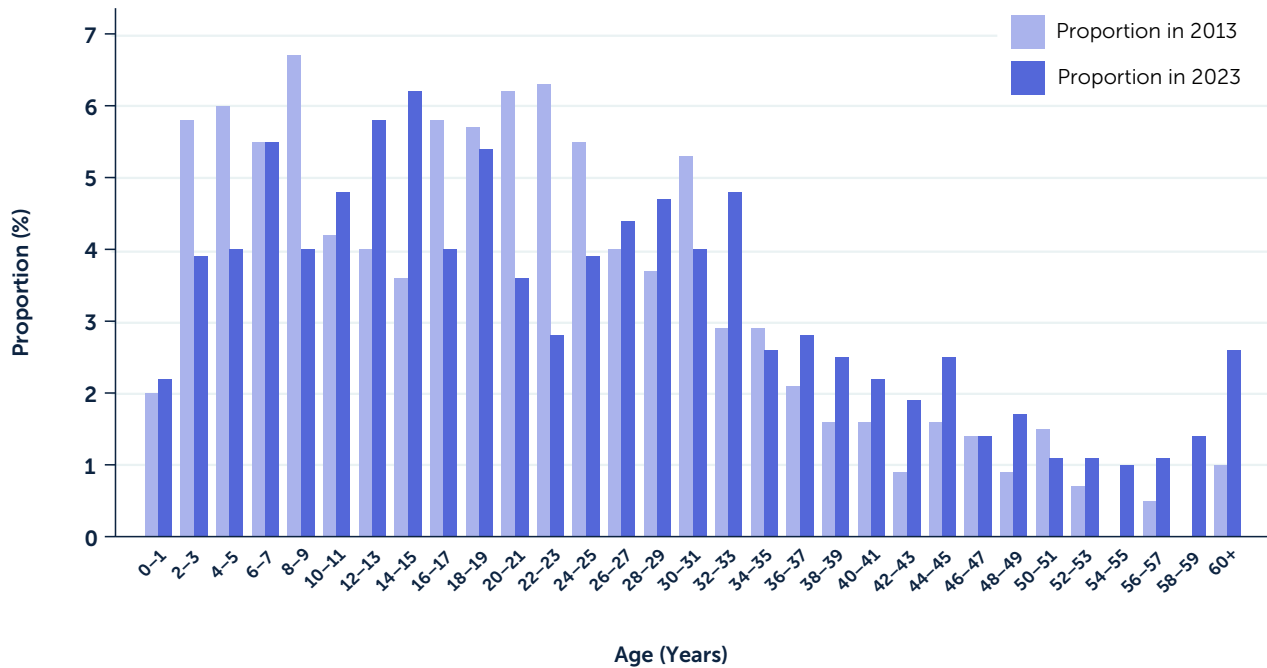
1.2 Age distribution by sex

The following chart shows the mix of ages by sex in the CF population in Scotland.



| Age | All; n(%) | Females; n(%) | Males; n(%) |
|---------|------------|---------------|-------------|
| 0-3 | 44 (6.1) | 21 (6.3) | 23 (5.9) |
| 4-7 | 69 (9.5) | 28 (8.4) | 41 (10.5) |
| 8-11 | 64 (8.9) | 33 (9.9) | 31 (7.9) |
| 12-15 | 87 (12.0) | 39 (11.7) | 48 (12.3) |
| 16-19 | 68 (9.4) | 27 (8.1) | 41 (10.5) |
| 20-23 | 46 (6.4) | 26 (7.8) | 20 (5.1) |
| 24-27 | 60 (8.3) | 30 (9.0) | 30 (7.7) |
| 28-31 | 63 (8.7) | 32 (9.6) | 31 (7.9) |
| 32-35 | 54 (7.5) | 25 (7.5) | 29 (7.4) |
| 36-39 | 38 (5.3) | 18 (5.4) | 20 (5.1) |
| 40-43 | 30 (4.1) | 12 (3.6) | 18 (4.6) |
| 44-47 | 28 (3.9) | 10 (3.0) | 18 (4.6) |
| 48-51 | 20 (2.8) | 10 (3.0) | 10 (2.6) |
| 52-55 | 15 (2.1) | 6 (1.8) | 9 (2.3) |
| 56-59 | 18 (2.5) | 7 (2.1) | 11 (2.8) |
| 60-67 | 19 (2.6) | 9 (2.7) | 10 (2.6) |
| <16 | 264 (36.5) | 121 (36.3) | 143 (36.7) |
| ≥16 | 459 (63.5) | 212 (63.7) | 247 (63.3) |
| <18 | 293 (40.5) | 134 (40.2) | 159 (40.8) |
| ≥18 | 430 (59.5) | 199 (59.8) | 231 (59.2) |
| Overall | 723 | 333 | 390 |

1.3 Age distribution of the UK CF population in 2013 vs 2023 N=723 in 2023, N=810 in 2013



1.4 Ethnicity

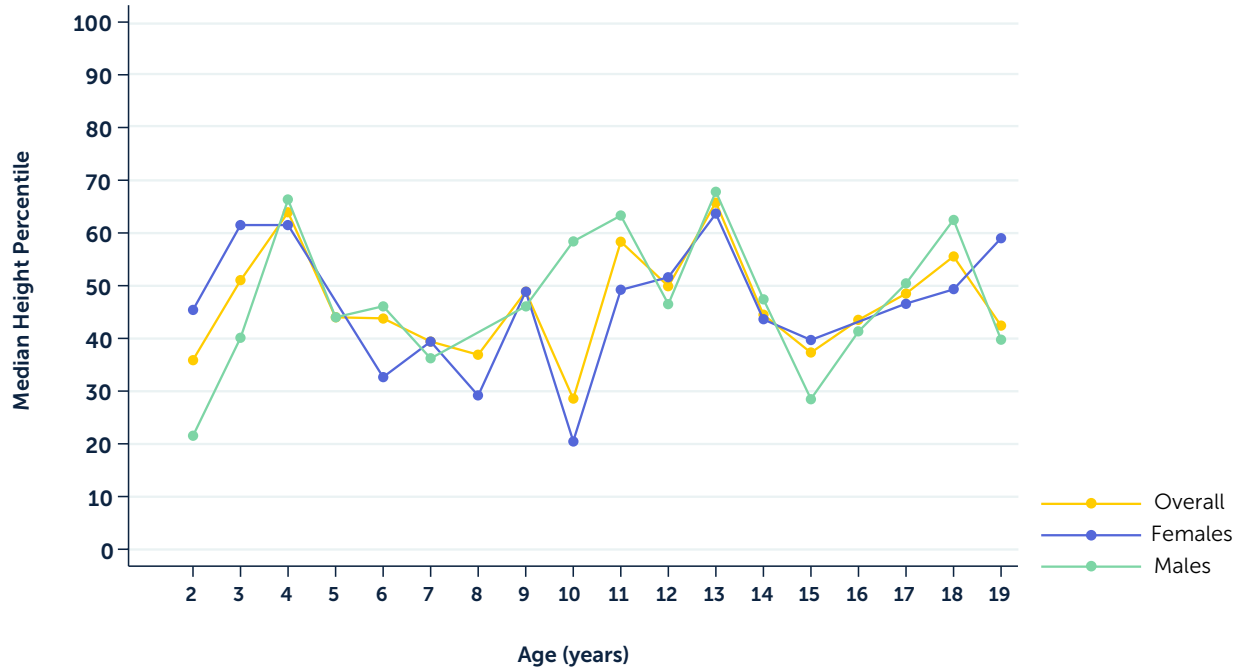
| Ethnicity n(%) | 2013 | 2018 | 2023 |
|--------------------------------|------------|------------|------------|
| Total | 810 | 819 | 723 |
| Total known¹ | 809 | 812 | 721 |
| White | 788 (97.4) | 796 (98.0) | 697 (96.7) |
| Asian | 14 (1.7) | 9 (1.1) | 18 (2.5) |
| Black | 0 (0.0) | 0 (0.0) | <5 |
| Mixed | <5 | <5 | -* |
| Other | -* | -* | 0 (0.0) |

1 Proportions are calculated from total known ethnicities.

* Redacted to adhere to statistical disclosure guidelines.

1.5 Height percentiles of children and young people (<20 years)¹ N=332

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 | 15 | 35.9 | 18.2-66.6 | 8 | 45.4 | 29.9-80.1 | 7 | 21.5 | 16.6-50.1 |
| 3 | 11 | 51.0 | 32.7-70.1 | 6 | 61.5 | 49.8-88.2 | 5 | 40.1 | 31.9-51.0 |
| 4 | 18 | 63.9 | 14.6-80.0 | 7 | 61.5 | 6.4-80.0 | 11 | 66.3 | 34.0-80.0 |
| 5 | 11 | 44.0 | 22.4-82.8 | <5 | -* | -* | 7 | 44.0 | 22.4-82.8 |
| 6 | 23 | 43.8 | 15.0-76.5 | 10 | 32.7 | 19.2-53.0 | 13 | 46.1 | 15.0-76.5 |
| 7 | 17 | 39.4 | 23.4-72.5 | 7 | 39.4 | 29.9-72.5 | 10 | 36.2 | 20.7-80.3 |
| 8 | 13 | 36.9 | 18.8-46.4 | 10 | 29.2 | 18.8-46.4 | <5 | -* | -* |
| 9 | 15 | 48.9 | 26.6-78.2 | 7 | 48.9 | 27.1-83.9 | 8 | 46.1 | 12.1-77.2 |
| 10 | 16 | 28.5 | 17.5-64.4 | 9 | 20.5 | 19.3-68.6 | 7 | 58.4 | 10.5-64.0 |
| 11 | 18 | 58.3 | 40.7-76.0 | 6 | 49.2 | 30.9-59.0 | 12 | 63.3 | 48.0-80.0 |
| 12 | 20 | 49.9 | 21.5-68.3 | 10 | 51.6 | 28.3-68.3 | 10 | 46.5 | 15.3-67.3 |
| 13 | 22 | 65.8 | 37.7-88.4 | 10 | 63.6 | 14.1-88.8 | 12 | 67.9 | 57.5-88.2 |
| 14 | 24 | 44.5 | 27.6-76.3 | 10 | 43.6 | 18.0-74.6 | 14 | 47.4 | 34.9-78.1 |
| 15 | 20 | 37.3 | 21.2-65.8 | 9 | 39.7 | 24.7-53.1 | 11 | 28.5 | 17.7-69.8 |
| 16 | 13 | 43.5 | 35.6-66.4 | <5 | -* | -* | 9 | 41.3 | 35.6-61.4 |
| 17 | 16 | 48.5 | 28.1-64.6 | 9 | 46.6 | 29.0-59.7 | 7 | 50.4 | 27.2-65.2 |
| 18 | 22 | 55.5 | 18.4-67.7 | 9 | 49.3 | 13.7-65.3 | 13 | 62.5 | 39.9-67.7 |
| 19 | 17 | 42.4 | 12.3-59.0 | 5 | 59.0 | 2.1-59.0 | 12 | 39.8 | 13.6-59.4 |
| Overall | 311** | 47.5 | 23.2-70.3 | 140 | 43.6 | 2.0-68.7 | 171 | 49.4 | 26.0-70.9 |

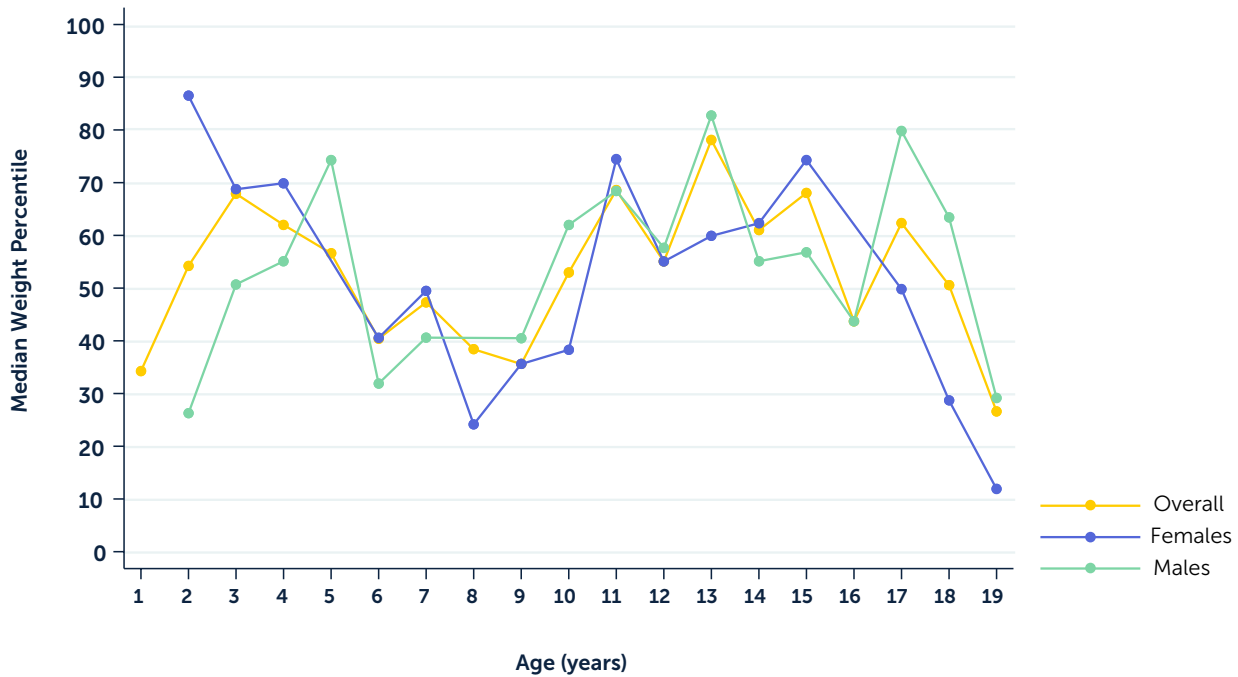
¹ Based on UK-WHO growth charts, 1990 (updated 1996).

* Redacted to adhere to statistical disclosure guidelines.

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

1.6 Weight percentiles of children and young people (<20 years)¹ N=332

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.



| Age | Overall | | | Female | | | Male | | |
|----------------|--------------|-------------|------------------|--------------|-------------|------------------|--------------|-------------|------------------|
| | n | Median | IQR | n | Median | IQR | n | Median | IQR |
| 1 | 12 | 34.2 | 9.3-59.7 | <5 | -* | -* | -* | . | 13.9-59.7 |
| 2 | 17 | 54.2 | 26.4-86.5 | 9 | 86.5 | 54.2-92.8 | 8 | 26.2 | 11.4-62.8 |
| 3 | 11 | 67.9 | 39.8-80.7 | 6 | 68.8 | 64.5-80.7 | 5 | 50.7 | 36.2-68.6 |
| 4 | 18 | 62.0 | 43.6-81.4 | 7 | 69.9 | 27.3-84.7 | 11 | 55.1 | 43.6-81.4 |
| 5 | 11 | 56.6 | 21.8-80.1 | <5 | -* | -* | -* | 74.3 | 21.8-85.5 |
| 6 | 23 | 40.4 | 10.4-62.2 | 10 | 40.6 | 31.6-56.9 | 13 | 31.9 | 5.6-62.2 |
| 7 | 17 | 47.3 | 32.6-82.1 | 7 | 49.5 | 33.4-96.9 | 10 | 40.6 | 28.7-82.1 |
| 8 | 13 | 38.4 | 20.6-71.2 | -* | 24.1 | 16.0-45.3 | <5 | -* | -* |
| 9 | 15 | 35.6 | 17.5-69.3 | 7 | 35.6 | 29.4-69.3 | 8 | 40.5 | 16.5-63.7 |
| 10 | 16 | 53.0 | 25.1-62.5 | 9 | 38.3 | 20.7-57.7 | 7 | 62.0 | 45.8-65.8 |
| 11 | 19 | 68.6 | 25.3-92.9 | 6 | 74.4 | 43.9-79.7 | 13 | 68.4 | 24.4-92.9 |
| 12 | 20 | 55.0 | 15.4-86.4 | 10 | 55.0 | 15.4-86.9 | 10 | 57.7 | 32.4-76.0 |
| 13 | 21 | 78.1 | 54.3-93.7 | 9 | 59.9 | 24.2-90.0 | 12 | 82.8 | 64.3-94.2 |
| 14 | 24 | 61.0 | 53.3-77.9 | 10 | 62.3 | 60.0-77.5 | 14 | 55.1 | 27.0-90.6 |
| 15 | 20 | 68.1 | 33.8-79.2 | 9 | 74.3 | 53.0-79.4 | 11 | 56.8 | 23.6-79.0 |
| 16 | 13 | 43.7 | 39.1-71.8 | <5 | -* | -* | -* | 43.7 | 42.5-82.7 |
| 17 | 16 | 62.3 | 43.6-85.1 | 9 | 49.8 | 41.0-70.4 | 7 | 79.8 | 50.3-88.7 |
| 18 | 22 | 50.6 | 27.3-79.8 | 9 | 28.7 | 8.9-63.9 | 13 | 63.4 | 28.2-88.7 |
| 19 | 17 | 26.6 | 11.9-41.8 | 5 | 11.9 | 3.0-34.0 | 12 | 29.2 | 16.4-46.7 |
| Overall | 325** | 54.3 | 27.1-79.7 | 144** | 53.2 | 28.4-78.4 | 181** | 55.1 | 26.4-81.2 |

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

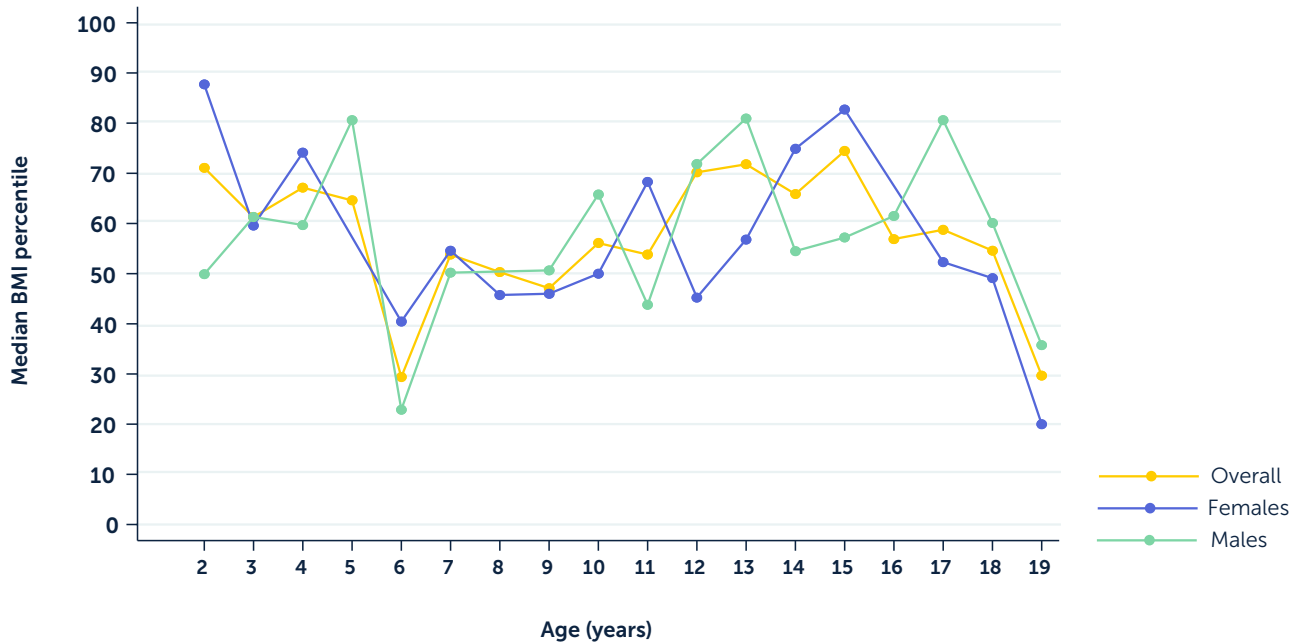
* Redacted to adhere to statistical disclosure guidelines.

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

1.7a Body Mass Index (BMI) percentiles in children and young people (<20 years)¹

N=332

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.



| Age | Overall | | | Female | | | Male | | |
|----------------|------------|-------------|------------------|------------|-------------|------------------|------------|-------------|------------------|
| | n | Median | IQR | n | Median | IQR | n | Median | IQR |
| 2 | 15 | 71.1 | 49.9-89.1 | 8 | 87.7 | 73.8-93.7 | 7 | 49.9 | 33.1-71.1 |
| 3 | 11 | 61.3 | 35.4-87.2 | 6 | 59.6 | 35.4-85.6 | 5 | 61.3 | 58.3-87.2 |
| 4 | 18 | 67.2 | 50.2-81.4 | 7 | 74.1 | 63.2-83.5 | 11 | 59.7 | 37.7-81.4 |
| 5 | 11 | 64.6 | 23.5-82.5 | <5 | -* | -* | -* | 80.6 | 64.6-88.4 |
| 6 | 23 | 29.4 | 22.5-68.9 | 10 | 40.4 | 24.7-68.9 | 13 | 22.9 | 19.9-68.2 |
| 7 | 17 | 53.8 | 42.1-64.2 | 7 | 54.6 | 48.9-98.2 | 10 | 50.2 | 33.2-56.2 |
| 8 | 13 | 50.3 | 22.6-68.3 | -* | 45.8 | 15.1-60.4 | <5 | -* | -* |
| 9 | 15 | 47.1 | 13.8-60.6 | 7 | 46.0 | 3.2-51.2 | 8 | 50.7 | 30.6-61.7 |
| 10 | 16 | 56.1 | 42.4-67.9 | 9 | 50.0 | 37.6-56.5 | 7 | 65.8 | 55.7-78.8 |
| 11 | 18 | 53.8 | 13.4-97.0 | 6 | 68.3 | 45.8-99.0 | 12 | 43.8 | 9.1-94.7 |
| 12 | 20 | 70.2 | 22.8-86.1 | 10 | 45.2 | 15.1-92.8 | 10 | 71.9 | 50.3-82.1 |
| 13 | 21 | 71.8 | 54.6-95.3 | 9 | 56.8 | 55.3-82.5 | 12 | 80.9 | 50.0-96.2 |
| 14 | 24 | 65.9 | 49.2-91.1 | 10 | 74.9 | 64.8-87.2 | 14 | 54.5 | 27.0-96.6 |
| 15 | 20 | 74.4 | 41.8-87.8 | 9 | 82.7 | 73.5-87.6 | 11 | 57.2 | 32.4-90.7 |
| 16 | 13 | 56.9 | 35.3-63.7 | <5 | -* | -* | -* | 61.5 | 35.3-87.5 |
| 17 | 16 | 58.8 | 35.7-91.3 | 9 | 52.3 | 33.4-63.0 | 7 | 80.6 | 54.6-94.7 |
| 18 | 22 | 54.6 | 26.8-80.6 | 9 | 49.1 | 23.4-80.0 | 13 | 60.1 | 29.7-89.5 |
| 19 | 17 | 29.7 | 16.8-47.7 | 5 | 20.0 | 6.6-22.1 | 12 | 35.8 | 21.8-53.5 |
| Overall | 310 | 56.8 | 30.5-82.9 | 139 | 56.3 | 29.8-82.7 | 171 | 58.0 | 30.5-83.4 |

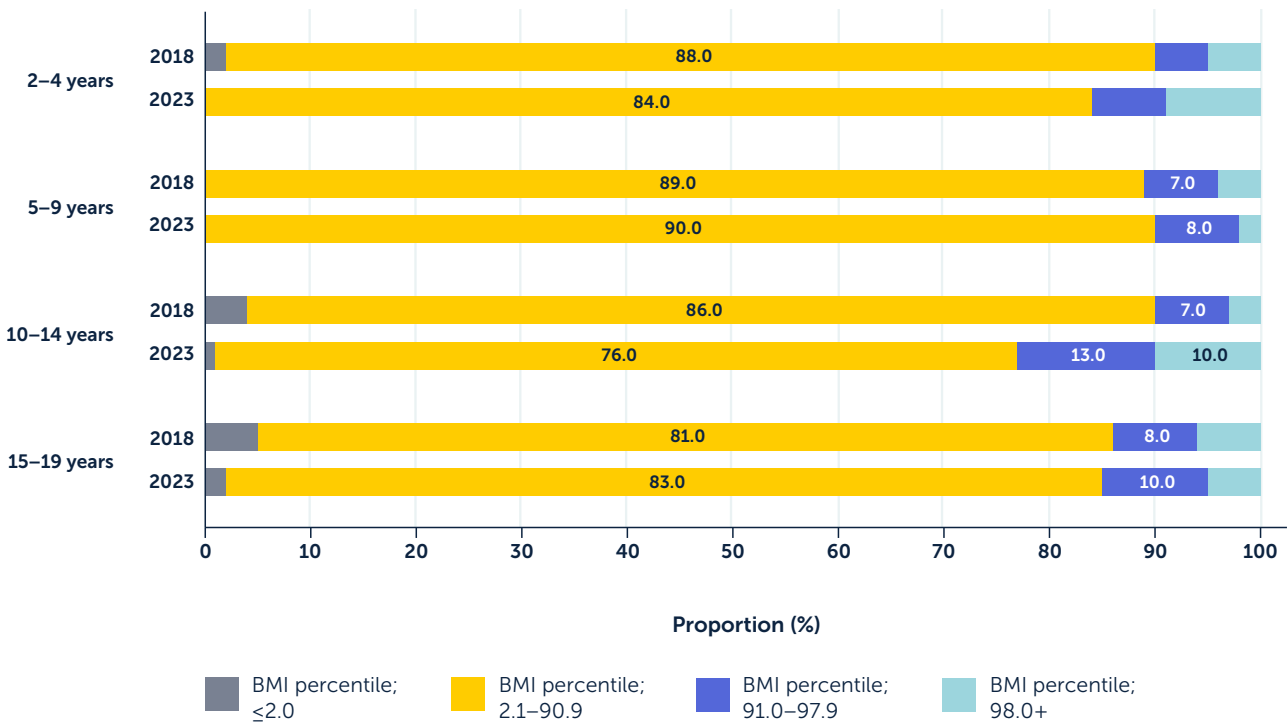
¹ Based on UK-WHO growth charts, 1990 (updated 1996).

* Redacted to adhere to statistical disclosure guidelines.

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

1.7b Body Mass Index (BMI) percentiles in children and young people (<20 years)¹ for 2018 and 2023

The following graph shows the change in BMI groups for children and young people with CF from 2018 to 2023.



| Age group | Year | Total number of people in each age group** | BMI category by age and year : n*(%) | | | |
|-------------|------|--|--------------------------------------|--------------------------|---------------------------|-----------------------|
| | | | BMI percentile; ≤2.0 | BMI percentile; 2.1-90.9 | BMI percentile; 91.0-97.9 | BMI percentile; 98.0+ |
| 2-4 years | 2018 | 43 | <5 | 38 (88.0) | <5 | <5 |
| | 2023 | 44 | 0 (0.0) | 37 (84.0) | <5 | <5 |
| 5-9 years | 2018 | 89 | 0 (0.0) | 79 (89.0) | 6 (7.0) | <5 |
| | 2023 | 79 | 0 (0.0) | 71 (90.0) | 6 (8.0) | <5 |
| 10-14 years | 2018 | 101 | <5 | 87 (86.0) | 7 (7.0) | <5 |
| | 2023 | 99 | <5 | 75 (76.0) | 13 (13.0) | 10 (10.0) |
| 15-19 years | 2018 | 63 | <5 | 51 (81.0) | 5 (8.0) | <5 |
| | 2023 | 88 | <5 | 73 (83.0) | 9 (10.0) | <5 |

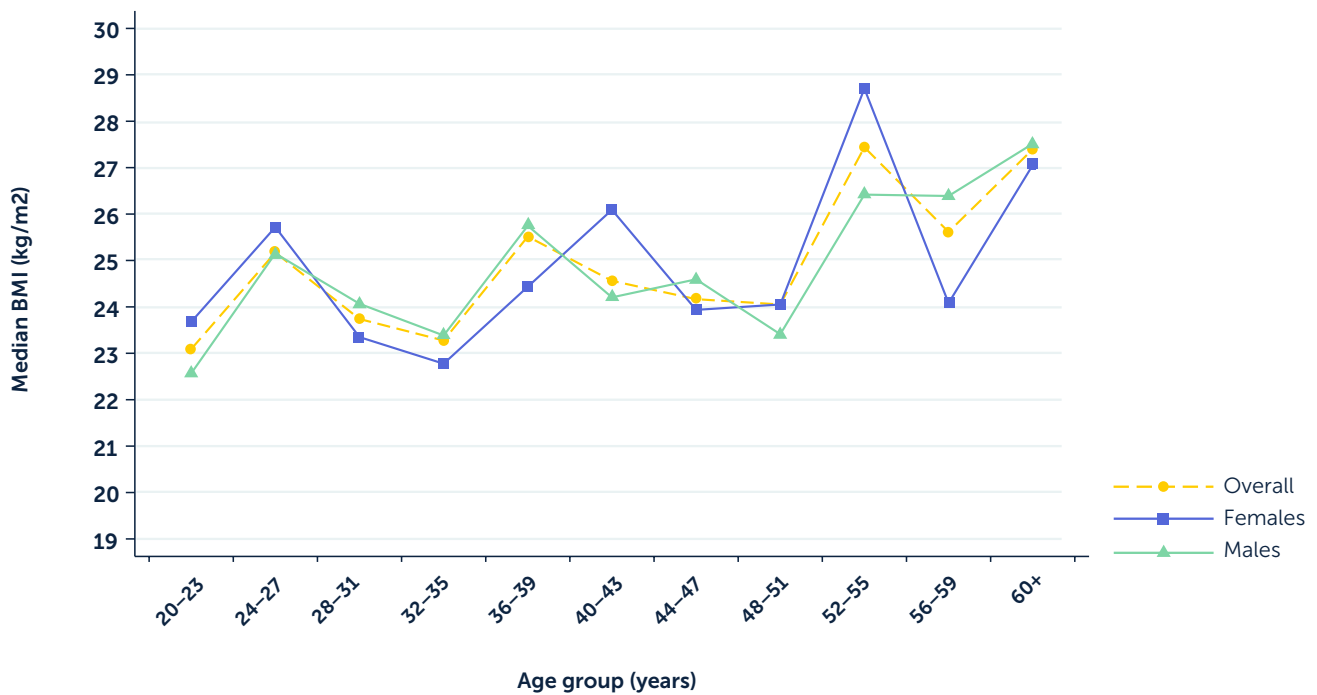
¹ Based on UK-WHO growth charts, 1990 (updated 1996).

* Redacted to adhere to statistical disclosure guidelines.

** And with non-missing data.

1.8a Body Mass Index (BMI) in adults (≥ 20 years) N=391

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

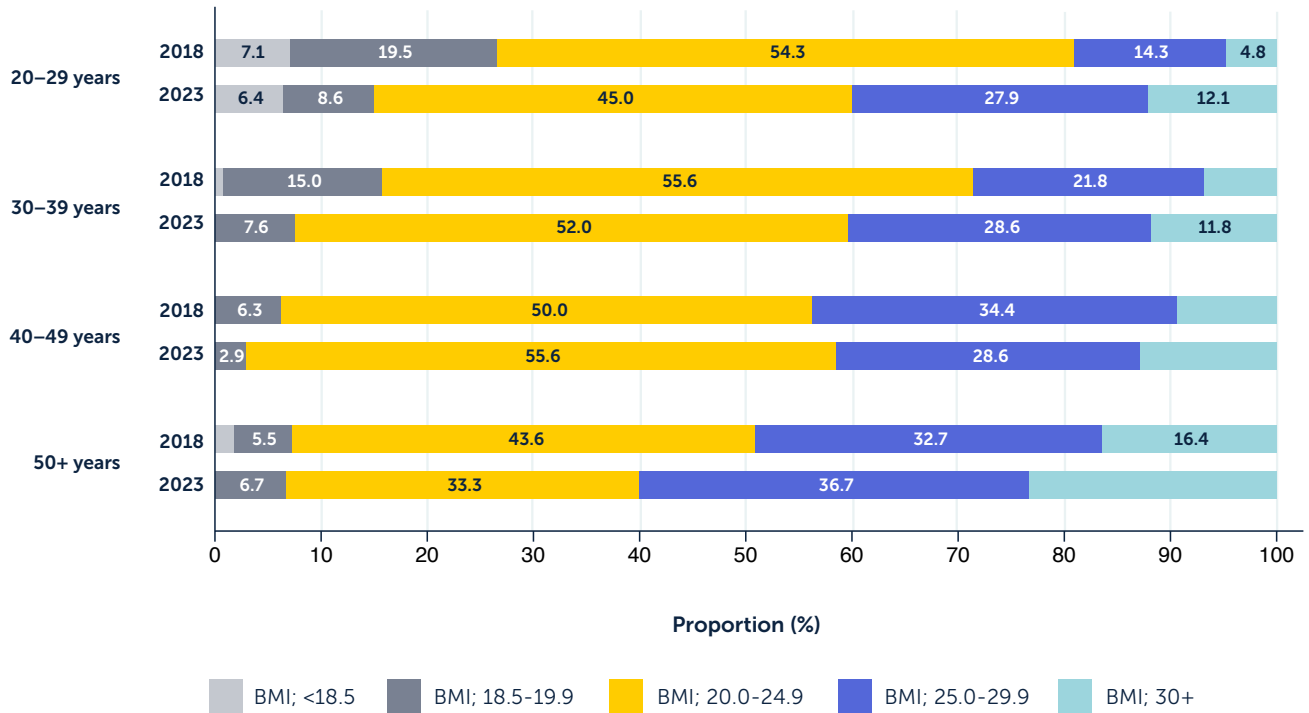


| Age | Overall | | | Female | | | Male | | |
|----------------|--------------|-------------|------------------|--------------|-------------|------------------|--------------|-------------|------------------|
| | n | Median | IQR | n | Median | IQR | n | Median | IQR |
| 20-23 | 46 | 23.1 | 20.9-25.5 | 26 | 23.7 | 20.6-25.5 | 20 | 22.6 | 20.9-25.5 |
| 24-27 | 60 | 25.2 | 21.9-28.8 | 30 | 25.7 | 21.7-29.4 | 30 | 25.2 | 22.1-28.3 |
| 28-31 | 63 | 23.7 | 22.1-26.0 | 32 | 23.3 | 21.7-26.1 | 31 | 24.1 | 22.1-25.7 |
| 32-35 | 53 | 23.3 | 21.4-25.7 | 24 | 22.8 | 20.8-26.0 | 29 | 23.4 | 21.8-25.5 |
| 36-39 | 37 | 25.5 | 22.2-28.4 | 17 | 24.4 | 22.0-27.4 | 20 | 25.7 | 23.4-29.3 |
| 40-43 | 30 | 24.6 | 21.8-28.7 | 12 | 26.1 | 22.7-30.7 | 18 | 24.2 | 21.8-28.2 |
| 44-47 | 28 | 24.2 | 22.8-26.5 | 10 | 23.9 | 21.7-26.3 | 18 | 24.6 | 23.0-26.7 |
| 48-51 | 20 | 24.1 | 21.7-26.6 | 10 | 24.1 | 22.8-25.5 | 10 | 23.4 | 21.2-28.3 |
| 52-55 | 15 | 27.4 | 23.7-30.6 | 6 | 28.7 | 23.9-30.6 | 9 | 26.4 | 23.7-30.0 |
| 56-59 | 18 | 25.6 | 22.2-28.4 | 7 | 24.1 | 22.1-30.5 | 11 | 26.4 | 24.9-28.4 |
| 60+ | 19 | 27.4 | 24.4-32.2 | 9 | 27.1 | 24.4-34.2 | 10 | 27.5 | 25.2-29.5 |
| Overall | 389** | 24.3 | 22.0-27.6 | 183** | 24.2 | 21.8-27.5 | 206** | 24.4 | 22.1-27.9 |

** Number with non-missing data.

1.8b Body Mass Index (BMI) in adults for 2018 and 2023

The following graph shows the change in the proportion of people in each BMI group in 2018 and 2023.



| Age group | Year | Total number of people in each age group** | BMI category by age and year : n*(%) | | | | |
|-------------|------|--|--------------------------------------|----------------|----------------|----------------|-----------|
| | | | BMI; <18.5 | BMI; 18.5-19.9 | BMI; 20.0-24.9 | BMI; 25.0-29.9 | BMI; 30+ |
| 20-29 years | 2018 | 210 | 15 (7.1) | 41 (19.5) | 114 (54.3) | 30 (14.3) | 10 (4.8) |
| | 2023 | 140 | 9 (6.4) | 12 (8.6) | 63 (45.0) | 39 (27.9) | 17 (12.1) |
| 30-39 years | 2018 | 133 | <5 | 20 (15.0) | 74 (55.6) | 29 (21.8) | -* |
| | 2023 | 119 | 0 (0.0) | 9 (7.6) | 62 (52.1) | 34 (28.6) | 14 (11.8) |
| 40-49 years | 2018 | 64 | 0 (0.0) | <5 | 32 (50.0) | 22 (34.4) | -* |
| | 2023 | 70 | 0 (0.0) | <5 | 39 (55.7) | 20 (28.6) | -* |
| 50+ years | 2018 | 55 | <5 | <5 | 24 (43.6) | 18 (32.7) | 9 (16.4) |
| | 2023 | 60 | 0 (0.0) | <5 | 20 (33.3) | 22 (36.7) | -* |

** And with non-missing data.

1.9 Education and employment in adults (≥ 16 years)

N=459

The following table shows how people with CF reported their education and employment status in 2023. Please note that the groups are not mutually exclusive; for example, someone may be a student as well as working part-time.

| | Overall numbers of patients; n(%) | Male; n(%) | Female; n(%) |
|--|-----------------------------------|-------------|--------------|
| Numbers of patients; n | 459 | 247 | 212 |
| Number who completed questionnaire; n(%) | 459 (100.0) | 247 (100.0) | 212 (100.0) |
| Full-time employment; n(%) | 188 (41.0) | 123 (49.8) | 65 (30.7) |
| Part-time employment; n(%) | 82 (17.9) | 27 (10.9) | 55 (25.9) |
| Student; n(%) | 83 (18.1) | 41 (16.6) | 42 (19.8) |
| Homemaker; n(%) | 11 (2.4) | <5 | 10 (4.7) |
| Unemployed; n(%) | 67 (14.6) | 38 (15.4) | 29 (13.7) |
| Disabled; n(%) | <5 | <5 | <5 |
| Retired; n(%) | 17 (3.7) | 13 (5.3) | <5 |
| Volunteer; n(%) | <5 | <5 | <5 |
| Unknown entered; n(%) | 7 (1.5) | <5 | <5 |
| No. in work or study; n(%) | 353 (76.9) | 191 (77.3) | 162 (76.4) |

* Redacted to adhere to statistical disclosure guidelines

1.10 Parenthood

| | 2020 | 2021 | 2022 | 2023 |
|-----------------------------------|------|------|------|------|
| Women with CF who had babies; n | <5 | 7 | 8 | 5 |
| Men with CF who became fathers; n | 0 | <5 | <5 | <5 |



Five women with cystic fibrosis had babies in Scotland during 2023

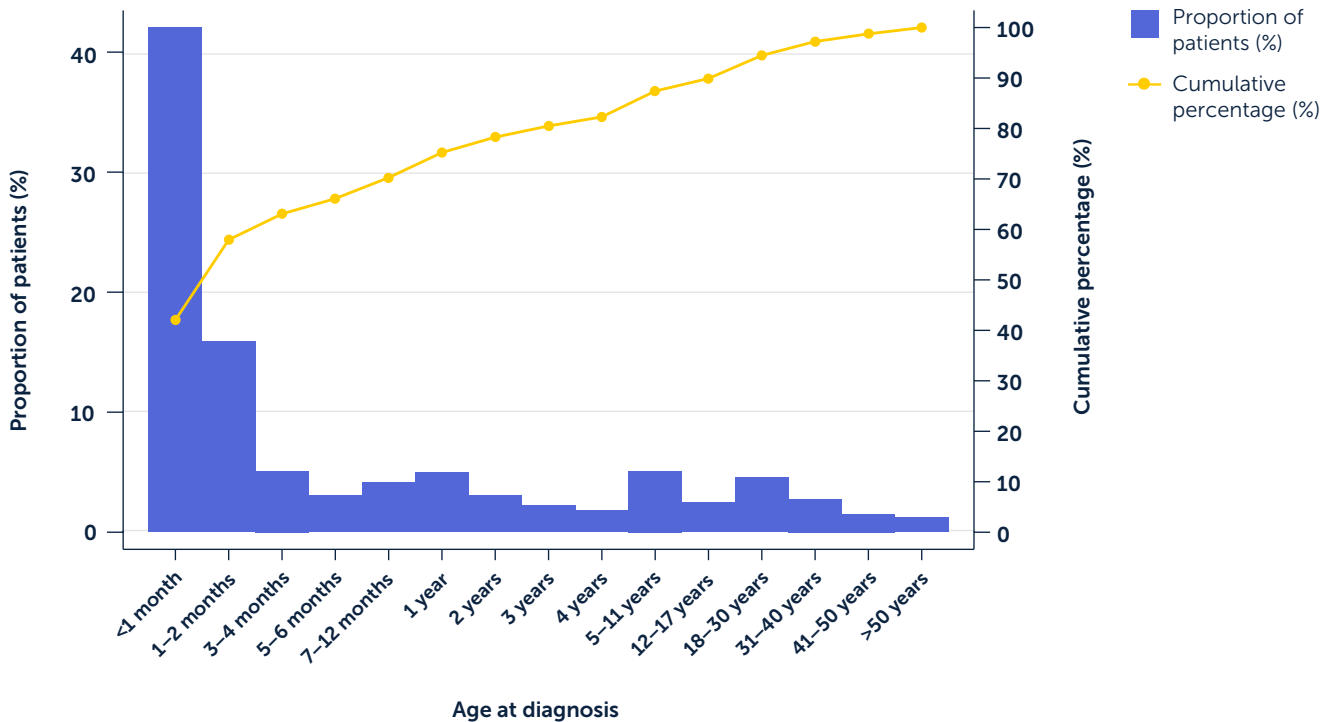


Fewer than five men with cystic fibrosis became fathers in Scotland during 2023

Diagnosis of cystic fibrosis

1.11 Age at diagnosis

N=723



The median age at diagnosis for patients aged under 16 in 2023 is **21 days**.

Newborn screening for CF has been done routinely in the whole of the UK since mid-2007. It is part of the heel-prick blood-spot testing done at five to seven days of age. The blood sample is tested for a number of conditions, including cystic fibrosis. This means that more babies born after 2007 receive an early diagnosis than those born before.

A total of **nine patients** born in 2023 were identified by newborn screening (including those without complete data).

79 (10.9%) of Scottish CF patients were diagnosed at age 16 or over. Five new CF diagnoses were recorded in Scotland for people aged 16 or over during 2023.

1.12 Mode of presentation

The following tables show the top five most frequent modes of presentation for those diagnosed between 2013–2023 and those born between 2013–2023, excluding those recorded as being diagnosed through newborn screening (NBS) or genotype.

| | All patients diagnosed 2013–2023 | Age <16 at diagnosis | Age ≥16 at diagnosis |
|--|----------------------------------|---------------------------------------|------------------------------------|
| Total patients | 187 | 154 | 33 |
| Number diagnosed by newborn screening | 134 | 134 | 0 |
| Total non-NBS or Genotype | 53 | 20 | 33 |
| Presentation Type** (for people not diagnosed by NBS or genotype) | Overall (n=53) | Age <16 at diagnosis (n=20) | Age ≥16 at diagnosis (n=33) |
| Persistent or acute respiratory infection | 14 (26.4) | <5 | 11 (33.3) |
| Meconium ileus | 10 (18.9) | 10 (50.0) | 0 (0.0) |
| Bronchiectasis | 8 (15.1) | 0 (0.0) | 8 (24.2) |
| Family history | 5 (9.4) | <5 | <5 |
| Unknown | <5 | 0 (0.0) | <5 |

| | All patients born 2013–2023 |
|--|-----------------------------|
| Total patients | 149 |
| Number diagnosed by newborn screening or genotype | 131 |
| Total non-NBS or Genotype | 18 |
| Presentation Type** (for people not diagnosed by NBS or genotype) | Overall (n=18) |
| Meconium ileus | 10 (55.6) |
| Persistent or acute respiratory infection | <5 |
| Failure to thrive/malnutrition | <5 |
| Family history | <5 |
| Prenatal | <5 |

* Redacted to adhere to statistical disclosure guidelines

** percentages may not total to 100 because (a) only top five presentation types are reported and (b) multiple presentation types can be selected.

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 FEV₁; the Forced Expiratory Volume of air in the first second of a forced exhaled breath. In this report, an FEV₁% predicted is based on the FEV₁ we would expect for a person without CF of the same age, gender, height, and ethnicity.

A person with CF who has FEV₁% 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 FEV₁% predicted of 50% breathes out half the volume of air as a comparable person without cystic fibrosis.

For people with CF, an FEV₁% 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 FEV₁ target, based on their own lung function results and trends.

An aim of CF care is to prevent FEV₁% 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 FEV₁% predicted values shown in this report are calculated using an equation called Global Lungs Initiative, or 'GLI'¹.

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

1.13 FEV₁% predicted (GLI equations) at annual review in patients aged six years and older who have not had a lung transplant N=628

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.

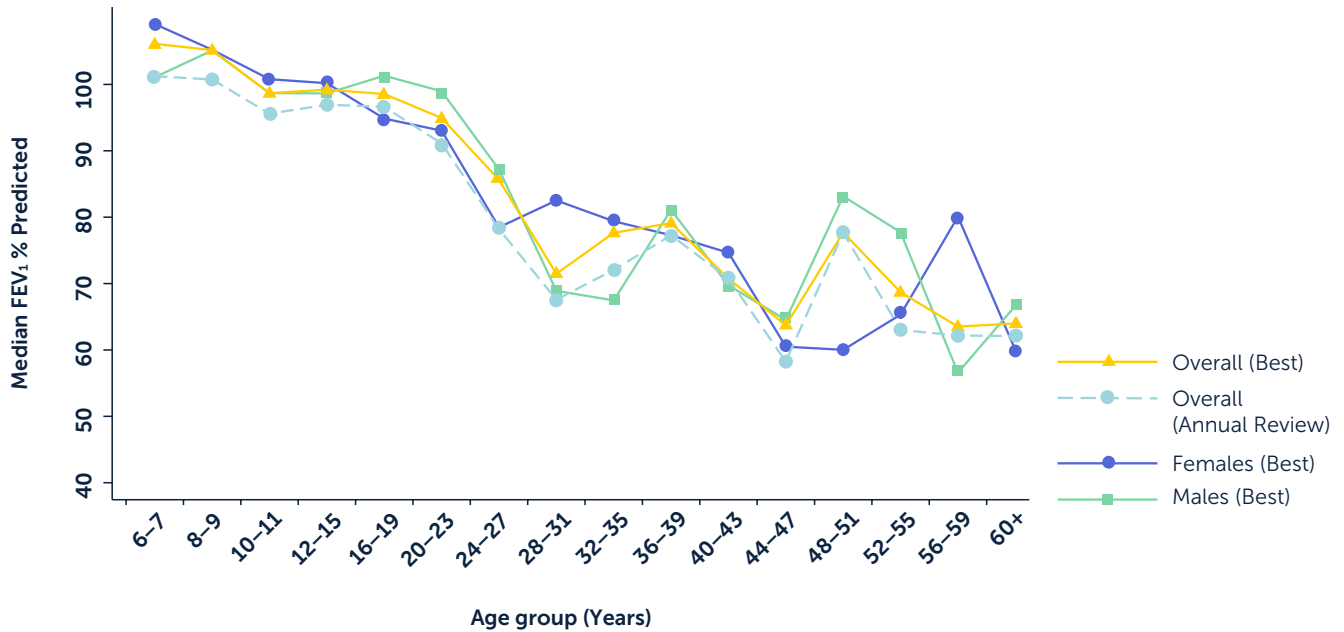
| Age (yrs) | Overall | | | Female | | | Male | | |
|----------------|--------------|-------------|-------------------|------------|-------------|------------------|------------|-------------|-------------------|
| | n | Median | IQR | n | Median | IQR | n | Median | IQR |
| 6-7 | 37 | 101.2 | 91.2-114.6 | 15 | 103.9 | 94.9-116.8 | 22 | 97.3 | 89.9-111.4 |
| 8-9 | 28 | 100.7 | 95.9-109.4 | 17 | 99.9 | 96.3-106.8 | 11 | 102.8 | 93.6-113.5 |
| 10-11 | 32 | 95.6 | 84.8-101.2 | 14 | 94.7 | 91.0-100.8 | 18 | 96.7 | 83.8-101.6 |
| 12-15 | 83 | 96.9 | 88.1-106.3 | 38 | 97.5 | 89.3-109.7 | 45 | 95.7 | 88.1-104.2 |
| 16-19 | 64 | 96.6 | 86.6-104.3 | 24 | 90.6 | 84.6-98.9 | 40 | 99.0 | 89.3-106.5 |
| 20-23 | 46 | 91.1 | 63.6-103.5 | 26 | 89.6 | 63.6-103.6 | 20 | 92.8 | 58.7-101.7 |
| 24-27 | 58 | 78.2 | 61.5-96.0 | 29 | 74.0 | 56.8-95.9 | 29 | 80.9 | 70.8-96.0 |
| 28-31 | 62 | 67.6 | 43.3-88.3 | 32 | 67.1 | 42.1-89.6 | 30 | 67.8 | 48.7-81.9 |
| 32-35 | 50 | 72.1 | 56.1-85.6 | 23 | 75.3 | 65.3-83.7 | 27 | 65.6 | 46.0-91.9 |
| 36-39 | 35 | 77.3 | 48.1-89.6 | 15 | 72.1 | 48.1-85.9 | 20 | 79.1 | 52.4-91.5 |
| 40-43 | 24 | 70.8 | 55.0-83.5 | 10 | 73.6 | 54.8-80.3 | 14 | 67.3 | 55.2-87.1 |
| 44-47 | 26 | 58.3 | 51.3-84.2 | 9 | 57.8 | 51.3-93.7 | 17 | 58.3 | 52.7-81.9 |
| 48-51 | 17 | 77.7 | 41.3-87.0 | 8 | 57.5 | 39.2-80.1 | 9 | 83.2 | 71.3-94.2 |
| 52-55 | 15 | 63.0 | 57.8-91.0 | 6 | 61.8 | 57.8-91.0 | 9 | 68.7 | 61.4-83.4 |
| 56-59 | 14 | 62.2 | 41.2-80.1 | 6 | 77.9 | 63.5-80.1 | 8 | 53.8 | 38.1-71.4 |
| 60-63 | 11 | 55.6 | 45.6-67.8 | -* | 55.6 | 45.6-67.8 | <5 | - | - |
| 64-67 | 186 | 97.6 | 89.4-107.0 | 85 | 98.8 | 90.8-109.7 | 101 | 96.6 | 86.9-106.3 |
| 68+ | 428 | 78.3 | 56.3-94.4 | 196 | 77.0 | 56.6-91.3 | 232 | 79.3 | 56.1-96.4 |
| <16 | 206 | 97.6 | 89.4-107.0 | 94 | 97.8 | 90.0-107.9 | 112 | 97.5 | 89.3-107.0 |
| ≥16 | 396 | 77.6 | 55.1-93.0 | 185 | 76.0 | 55.6-91.4 | 211 | 78.3 | 53.1-95.3 |
| Overall | 602** | 86.9 | 66.6-100.3 | 279 | 85.9 | 66.1-99.9 | 323 | 87.2 | 67.0-100.7 |

* Redacted to adhere to statistical disclosure guidelines.

** Number with non-missing data.

1.14 Best FEV₁% predicted (GLI equations) in patients aged six years and older who have not had a lung transplant N=628

For the best FEV₁ calculation, where best FEV₁% was missing or less than the FEV₁% at annual review, the annual review FEV₁% was used.



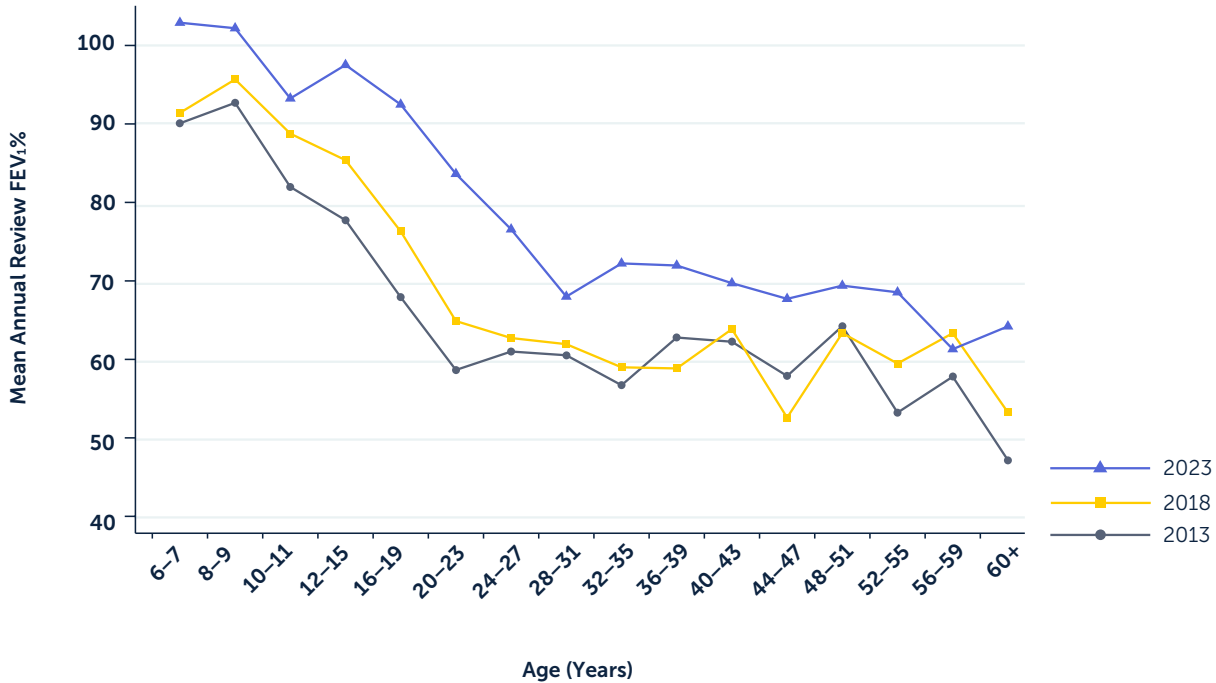
| Age (yrs) | Overall | | | Female | | | Male | | |
|----------------|--------------|-------------|-------------------|------------|-------------|-------------------|------------|-------------|-------------------|
| | n | Median | IQR | n | Median | IQR | n | Median | IQR |
| 6-7 | 40 | 106.1 | 96.0-114.4 | 17 | 109.1 | 104.2-114.6 | 23 | 101.1 | 93.8-114.3 |
| 8-9 | 28 | 105.2 | 98.6-112.1 | 17 | 105.2 | 98.7-109.5 | 11 | 105.1 | 96.6-113.5 |
| 10-11 | 33 | 98.7 | 93.0-104.6 | 14 | 100.8 | 91.0-105.1 | 19 | 98.7 | 93.5-103.6 |
| 12-15 | 86 | 99.2 | 90.4-108.8 | 39 | 100.2 | 91.0-110.6 | 47 | 98.7 | 89.4-107.2 |
| 16-19 | 68 | 98.6 | 89.3-106.9 | 27 | 94.8 | 87.1-102.5 | 41 | 101.3 | 93.0-110.3 |
| 20-23 | 46 | 94.9 | 75.2-104.7 | 26 | 93.0 | 84.9-103.6 | 20 | 99.0 | 67.8-107.2 |
| 24-27 | 59 | 85.6 | 68.9-96.4 | 29 | 78.5 | 61.2-95.9 | 30 | 87.2 | 77.6-96.4 |
| 28-31 | 62 | 71.5 | 52.3-94.0 | 32 | 82.5 | 52.7-98.2 | 30 | 68.9 | 52.3-86.0 |
| 32-35 | 51 | 77.7 | 56.1-94.8 | 24 | 79.4 | 68.6-87.3 | 27 | 67.5 | 46.6-95.6 |
| 36-39 | 35 | 79.1 | 59.9-94.7 | 15 | 77.3 | 59.9-88.3 | 20 | 81.1 | 56.7-97.4 |
| 40-43 | 24 | 70.8 | 55.0-88.4 | 10 | 74.6 | 54.8-82.7 | 14 | 69.7 | 55.2-89.3 |
| 44-47 | 26 | 63.7 | 52.7-91.8 | 9 | 60.5 | 55.9-93.7 | 17 | 64.5 | 52.7-86.9 |
| 48-51 | 17 | 77.7 | 46.8-87.6 | 8 | 60.0 | 46.8-81.8 | 9 | 83.2 | 77.7-94.2 |
| 52-55 | 15 | 68.7 | 62.3-91.0 | 6 | 65.3 | 62.3-91.0 | 9 | 77.7 | 63.0-89.7 |
| 56-59 | 15 | 63.5 | 43.3-83.9 | 7 | 80.1 | 63.5-83.9 | 8 | 56.6 | 39.2-73.9 |
| 60+ | 18 | 64.0 | 53.6-77.4 | 8 | 60.0 | 53.0-74.7 | 10 | 66.7 | 55.2-86.3 |
| <16 | 187 | 101.1 | 93.0-110.6 | 87 | 104.1 | 93.5-111.2 | 100 | 100.4 | 92.6-109.9 |
| ≥16 | 436 | 82.9 | 61.5-98.2 | 201 | 82.7 | 63.6-95.9 | 235 | 83.1 | 59.2-99.5 |
| <18 | 216 | 100.9 | 92.6-109.9 | 100 | 102.1 | 92.1-110.0 | 116 | 100.6 | 92.8-109.9 |
| ≥18 | 206 | 88.8 | 67.5-100.8 | 101 | 89.4 | 64.4-100.7 | 105 | 87.4 | 68.1-101.3 |
| Overall | 623** | 91.0 | 70.4-103.1 | 288 | 90.8 | 71.2-103.4 | 335 | 92.0 | 68.7-102.8 |

* Redacted to adhere to statistical disclosure guidelines.

** Number with non-missing data.

1.15 FEV₁% predicted (GLI equations) over time in patients aged six years and older who have not had a lung transplant N=628 in 2023, N=698 in 2018, N=604 in 2013**

The chart below shows how FEV₁% in 2023 compares to Registry data from 2013 and 2018. 2013 is shown as a comparator year.



| Age (yrs) | n | 2013 mean FEV ₁ %: Mean (SD) | n | 2018 mean FEV ₁ %: Mean (SD) | n | 2023 mean FEV ₁ %: Mean (SD) | p-values (t-test)*** |
|-----------|-----|--|-----|--|-----|--|-------------------------|
| 6-7 | 45 | 90.1 (12.5) | 45 | 91.4 (13.7) | 37 | 102.9 (16.6) | <0.001 |
| 8-9 | 47 | 92.7 (10.6) | 39 | 95.7 (15.9) | 28 | 102.2 (10.0) | 0.061 |
| 10-11 | 29 | 82.0 (12.8) | 31 | 88.8 (15.8) | 32 | 93.3 (12.4) | 0.214 |
| 12-15 | 59 | 77.7 (21.5) | 90 | 85.4 (16.1) | 83 | 97.5 (13.9) | <0.001 |
| 16-19 | 97 | 67.9 (22.8) | 51 | 76.3 (22.4) | 64 | 92.5 (17.1) | <0.001 |
| 20-23 | 91 | 58.6 (23.7) | 90 | 64.9 (26.4) | 46 | 83.6 (25.3) | <0.001 |
| 24-27 | 71 | 61.0 (25.0) | 77 | 62.7 (23.8) | 58 | 76.6 (23.9) | 0.001 |
| 28-31 | 59 | 60.5 (24.1) | 62 | 61.9 (22.8) | 62 | 68.0 (24.8) | 0.160 |
| 32-35 | 36 | 56.7 (22.7) | 58 | 59.0 (20.5) | 50 | 72.2 (25.0) | 0.003 |
| 36-39 | 23 | 62.8 (24.0) | 34 | 58.8 (24.2) | 35 | 72.0 (22.6) | 0.023 |
| 40-43 | 15 | 62.2 (25.9) | 27 | 63.9 (26.8) | 24 | 69.7 (18.7) | 0.375 |
| 44-47 | 19 | 57.9 (21.6) | 14 | 52.6 (25.3) | 26 | 67.7 (26.9) | 0.091 |
| 48-51 | 13 | 64.2 (19.8) | 25 | 63.4 (20.2) | 17 | 69.4 (26.0) | 0.402 |
| 52-55 | 6 | 53.2 (16.7) | 20 | 59.5 (20.8) | 15 | 68.5 (23.2) | 0.232 |
| 56-59 | 6 | 57.8 (26.8) | 7 | 63.3 (21.1) | 14 | 61.3 (20.4) | **** |
| 60+ | 5 | 47.2 (23.9) | 12 | 53.2 (26.0) | 17 | 64.2 (20.4) | 0.214 |
| <16 | 180 | 85.4 (16.8) | 205 | 89.2 (15.9) | 180 | 98.6 (14.0) | N/A |
| ≥16 | 441 | 61.4 (23.6) | 477 | 63.2 (24.0) | 428 | 75.1 (24.5) | N/A |
| <18 | 227 | 82.4 (19.1) | 225 | 88.3 (16.5) | 206 | 97.9 (14.3) | N/A |
| ≥18 | 394 | 60.3 (23.6) | 457 | 62.5 (23.9) | 402 | 73.9 (24.5) | N/A |

* Redacted to adhere to statistical disclosure guidelines.

** Due to missing data, means are calculated from a population of 608 in 2023, 682 in 2018 and 621 in 2013.

*** t-test comparing 2023 with 2018. If the p-value is less than 0.05 then the difference in the mean is statistically significant.

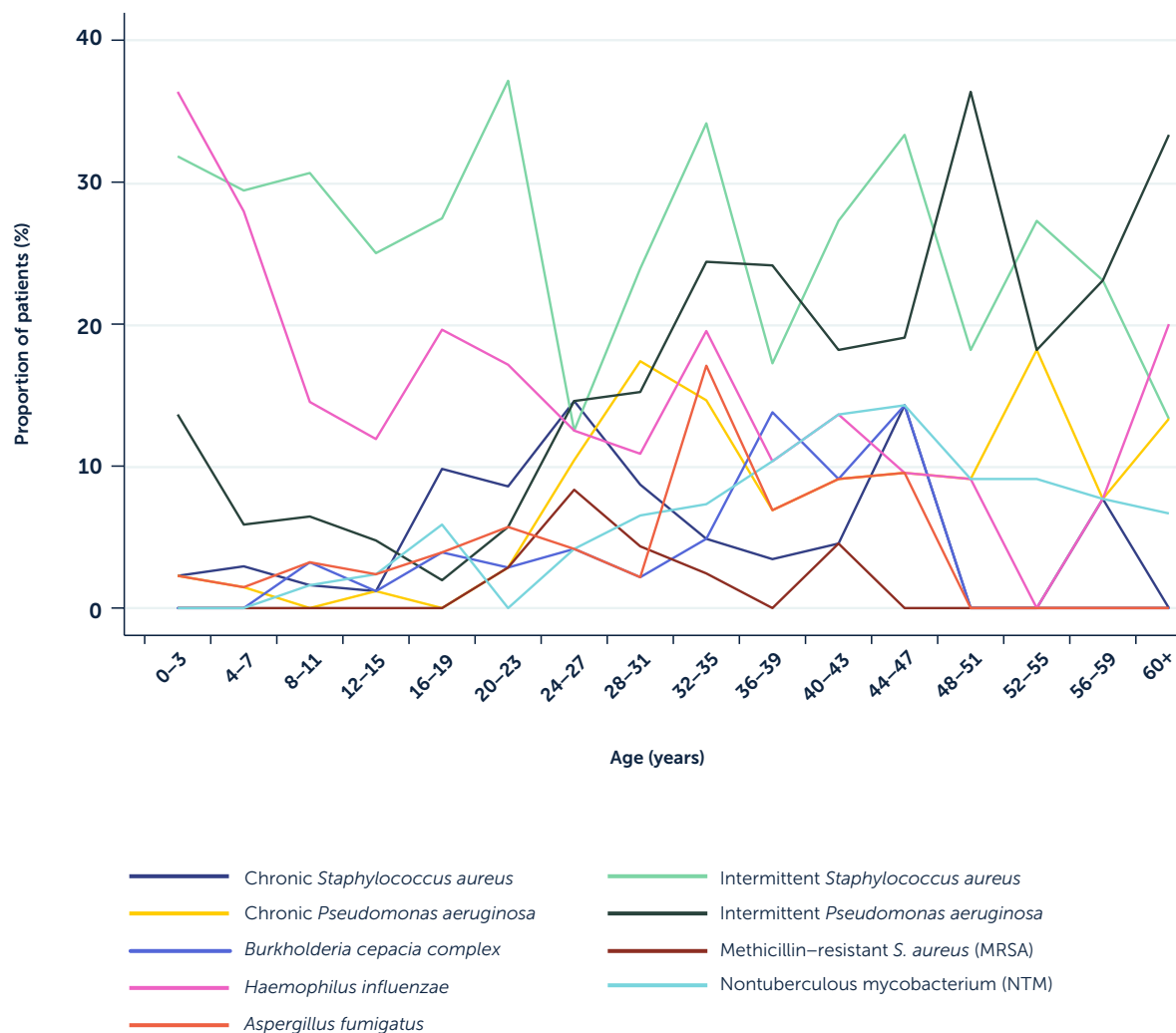
**** t-test not performed due to small numbers in these age groups.

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 data set.

1.16 Lung infections in 2023

N=601*



* Proportions are calculated from the number of patients with at least one sample taken in the relevant age group. Some proportions should be treated with caution due to the very small numbers of people with the infections in some age groups.

1.17 Lung infections in 2023

<16 years N=258; ≥16 years N=343

Infections in this table reflect bugs grown in the 12 months prior to the 2023 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 | 44 | 69 | 64 | 87 | 264 |
| Number who had culture taken* | 44 | 68 | 62 | 84 | 258 |
| Chronic <i>S. aureus</i> n(%) | <5 | <5 | <5 | <5 | 5 (5.0) |
| Intermittent <i>S. aureus</i> n(%) | 14 (14.0) | 20 (20.0) | 19 (19.0) | 21 (21.0) | 74 (74.0) |
| Chronic <i>P. aeruginosa</i> n(%) | <5 | <5 | 0 | <5 | <5 |
| Intermittent <i>P. aeruginosa</i> n(%) | 6 (6.0) | <5 | <5 | <5 | 18 (18.0) |
| <i>B. cepacia</i> complex n(%) | 0 | 0 | <5 | <5 | <5 |
| <i>B. cenocepacia</i> n(%) | 0 | 0 | 0 | 0 | 0 |
| <i>B. multivorans</i> n(%) | 0 | 0 | 0 | 0 | 0 |
| <i>B. cepacia</i> (other) n(%) | 0 | 0 | 0 | 0 | 0 |
| MRSA n(%) | 0 | 0 | 0 | 0 | 0 |
| <i>H. influenza</i> n(%) | 16 (16.0) | 19 (19.0) | 9 (9.0) | 10 (10.0) | 54 (54.0) |
| NTM n(%) | 0 | 0 | <5 | <5 | <5 |
| <i>Aspergillus fumigatus</i> n(%) | <5 | <5 | <5 | <5 | 6 (6.0) |

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

Lung infections in 2023 (contd.)

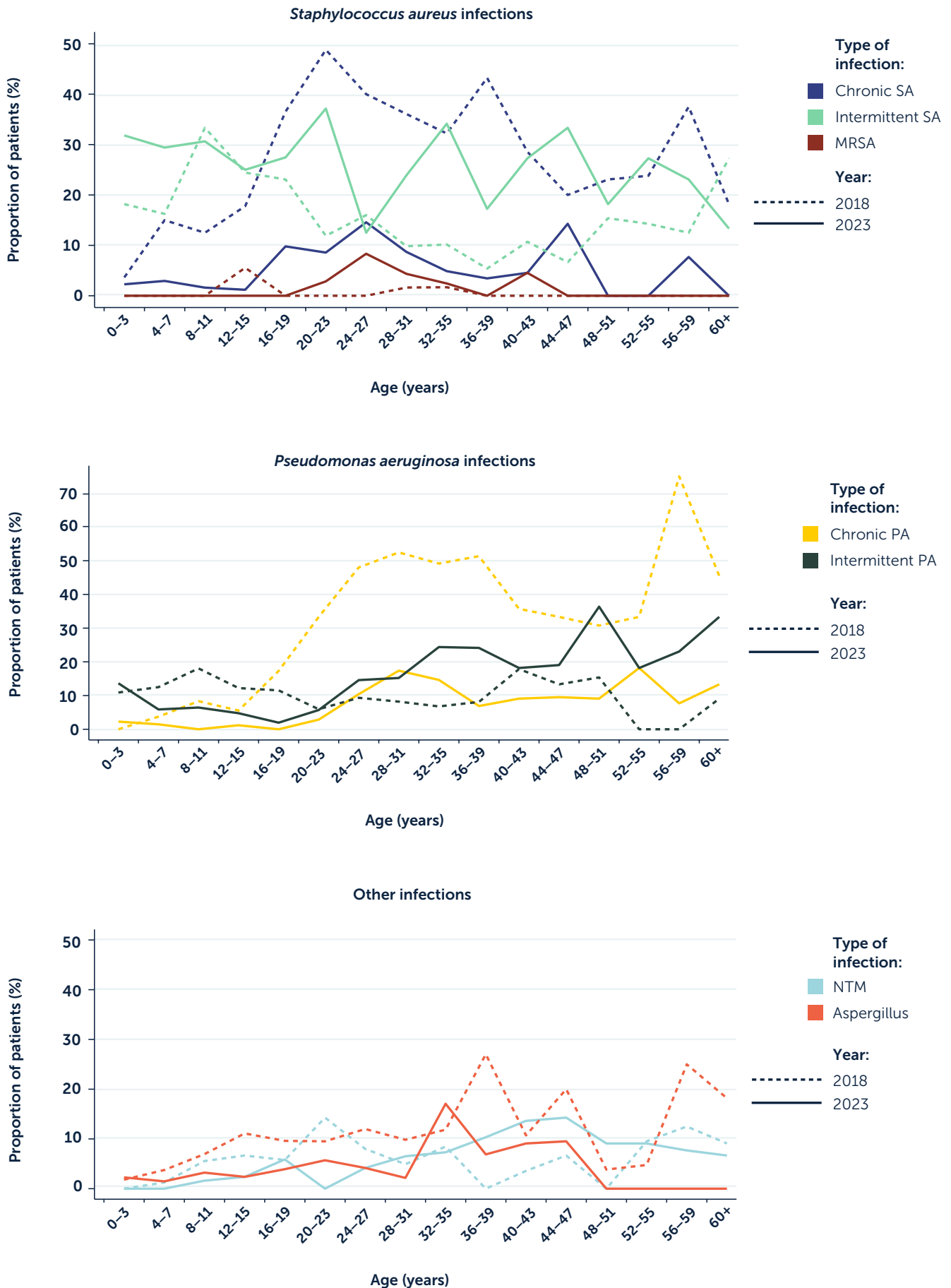
<16 years N=258; ≥16 years N=343

| | Adult Age Range (Years) | | | | | | Overall |
|--|-------------------------|-----------|---------|-----------|-----------|---------|--------------------|
| | 16-19 | 20-23 | 24-27 | 28-31 | 32-35 | 36-39 | Adults (≥16 years) |
| Number in age range | 68 | 46 | 60 | 63 | 54 | 38 | 459 |
| Number who had culture taken* | 51 | 35 | 48 | 46 | 41 | 29 | 343 |
| Chronic <i>S. aureus</i> n(%) | 5 (5.0) | <5 | 7 (7.0) | <5 | <5 | <5 | 27 (27.0) |
| Intermittent <i>S. aureus</i> n(%) | 14 (14.0) | 13 (13.0) | 6 (6.0) | 11 (11.0) | 14 (14.0) | 5 (5.0) | 86 (86.0) |
| Chronic <i>P. aeruginosa</i> n(%) | 0 | <5 | 5 (5.0) | 8 (8.0) | 6 (6.0) | <5 | 32 (32.0) |
| Intermittent <i>P. aeruginosa</i> n(%) | <5 | <5 | 7 (7.0) | 7 (7.0) | 10 (10.0) | 7 (7.0) | 56 (56.0) |
| <i>B. cepacia</i> complex n(%) | <5 | <5 | <5 | <5 | <5 | <5 | 17 (17.0) |
| <i>B. cenocepacia</i> n(%) | 0 | 0 | 0 | 0 | <5 | <5 | 5 (5.0) |
| <i>B. multivorans</i> n(%) | <5 | <5 | <5 | 0 | <5 | <5 | 10 (10.0) |
| <i>B. cepacia</i> (other) n(%) | 0 | 0 | 0 | <5 | 0 | 0 | <5 |
| MRSA n(%) | 0 | <5 | <5 | <5 | <5 | 0 | 9 (9.0) |
| <i>H. influenza</i> n(%) | 10 (10.0) | 6 (6.0) | 6 (6.0) | 5 (5.0) | 8 (8.0) | <5 | 48 (48.0) |
| NTM n(%) | <5 | 0 | <5 | <5 | <5 | <5 | 24 (24.0) |
| <i>Aspergillus</i> n(%) | <5 | <5 | <5 | <5 | 7 (7.0) | <5 | 20 (20.0) |

| | Adult Age Range (Years) | | | | | | Overall |
|--|-------------------------|---------|-------|-------|-------|---------|--------------------|
| | 40-43 | 44-47 | 48-51 | 52-55 | 56-59 | 60+ | Adults (≥16 years) |
| Number in age range | 30 | 28 | 20 | 15 | 18 | 19 | 459 |
| Number who had culture taken* | 22 | 21 | 11 | 11 | 13 | 15 | 343 |
| Chronic <i>S. aureus</i> n(%) | <5 | <5 | 0 | 0 | <5 | 0 | 27 (27.0) |
| Intermittent <i>S. aureus</i> n(%) | 6 (6.0) | 7 (7.0) | <5 | <5 | <5 | <5 | 86 (86.0) |
| Chronic <i>P. aeruginosa</i> n(%) | <5 | <5 | <5 | <5 | <5 | <5 | 32 (32.0) |
| Intermittent <i>P. aeruginosa</i> n(%) | <5 | <5 | <5 | <5 | <5 | 5 (5.0) | 56 (56.0) |
| <i>B. cepacia</i> complex n(%) | <5 | <5 | 0 | 0 | 0 | 0 | 17 (17.0) |
| <i>B. cenocepacia</i> n(%) | <5 | 0 | 0 | 0 | 0 | 0 | 5 (5.0) |
| <i>B. multivorans</i> n(%) | 0 | <5 | 0 | 0 | 0 | 0 | 10 (10.0) |
| <i>B. cepacia</i> (other) n(%) | 0 | 0 | 0 | 0 | 0 | 0 | <5 |
| MRSA n(%) | <5 | 0 | 0 | 0 | 0 | 0 | 9 (9.0) |
| <i>H. influenza</i> n(%) | <5 | <5 | <5 | 0 | <5 | <5 | 48 (48.0) |
| NTM n(%) | <5 | <5 | <5 | <5 | <5 | <5 | 24 (24.0) |
| <i>Aspergillus</i> n(%) | <5 | <5 | 0 | 0 | 0 | 0 | 20 (20.0) |

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

1.18 Lung infections in 2018 and 2023



1.19 Respiratory culture sample type

| Overall | 2018 | 2023 |
|--|------------|------------|
| Number of people with an annual review (n) | 819 | 723 |
| Number of people with at least 3 samples of any type taken n(%)* | 648 (79.1) | 411 (56.8) |
| Number of people with at least 1 sample of any type taken n(%)* | 774 (94.5) | 604 (83.5) |
| Sample type** | | |
| Sputum; n(%) | 571 (73.8) | 347 (57.5) |
| Cough; n(%) | 332 (42.9) | 379 (62.7) |
| Bronchoalveolar lavage; n(%) | 17 (2.2) | 19 (3.1) |
| | | |
| Age <16 years | 2018 | 2023 |
| Number of people with an annual review (n) | 304 | 264 |
| Number of people with at least 3 samples of any type taken n(%)* | 280 (92.1) | 249 (94.3) |
| Number of people with at least 1 sample of any type taken n(%)* | 297 (97.7) | 261 (98.9) |
| Sample type** | | |
| Sputum; n(%) | 105 (35.4) | 57 (21.8) |
| Cough; n(%) | 284 (95.6) | 259 (99.2) |
| Bronchoalveolar lavage; n(%) | 16 (5.4) | 14 (5.4) |
| | | |
| Age >=16 years | 2018 | 2023 |
| Number of people with an annual review (n) | 515 | 459 |
| Number of people with at least 3 samples of any type taken n(%)* | 368 (71.5) | 162 (35.3) |
| Number of people with at least 1 sample of any type taken n(%)* | 477 (92.6) | 343 (74.7) |
| Sample type** | | |
| Sputum; n(%) | 466 (97.7) | 290 (84.5) |
| Cough; n(%) | 48 (10.1) | 120 (35.0) |
| Bronchoalveolar lavage; n(%) | <5 | <5 |

1.20 Non-tuberculous mycobacteria (NTM) or atypical mycobacteria

NTM 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.

| | 2021 (n=777) | 2022 (n=727) | 2023 (n=723) |
|--|-----------------|-----------------|-----------------|
| NTM prevalence; n(%) | 28 (3.6) | 24 (3.3) | 27 (3.7) |
| On NTM treatment in the given year; n (% of NTM prevalence in given year) | 11 (39.3) | 5 (20.8) | 10 (37.0) |
| NTM incidence | 13 (1.8) | 17 (2.5) | 18 (2.6) |
| <i>M. abscessus</i> prevalence | 14 (1.8) | <5 | 5 (0.7) |
| <i>M. abscessus</i> incidence | <5 | <5 | <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%.

Complications

1.21 Complications in 2023

The number shown is for a complication that has been present in the preceding 12 months.

| Complications | Overall | <16 years | ≥16 years |
|---|------------|-----------|------------|
| Respiratory related | | | |
| Nasal polyps requiring surgery | 15 (1.9) | <5 | -* |
| Sinus disease | 82 (10.2) | 0 | 82 (17.9) |
| Asthma | 45 (5.6) | <5 | -* |
| ABPA | 18 (2.2) | <5 | -* |
| Haemoptysis (massive, severe and/or moderate) | 8 (1.0) | 0 | 8 (1.7) |
| Massive haemoptysis | <5 | 0 | <5 |
| Pneumothorax requiring chest tube | 0 | 0 | 0 |
| Cardiac complications | | | |
| Tachyarrhythmia | 0 | 0 | 0 |
| Bradycardia | 0 | 0 | 0 |
| Cardiac arrest | <5 | 0 | <5 |
| Cardiomyopathy | <5 | 0 | <5 |
| Congenital heart disease | 0 | 0 | 0 |
| Heart failure | 0 | 0 | 0 |
| Ischaemic heart disease | 0 | 0 | 0 |
| Valvular disease | 0 | 0 | 0 |
| Other | <5 | 0 | <5 |
| Pancreas and hepatobiliary disease | | | |
| Raised liver enzymes | 44 (5.5) | 6 (2.3) | 38 (8.3) |
| Liver disease | 144 (17.9) | 25 (9.5) | 119 (25.9) |
| Cirrhosis with no portal hypertension | 8 (1.0) | <5 | -* |
| Cirrhosis with portal hypertension | 20 (2.5) | <5 | -* |
| Gall bladder disease requiring surgery | 11 (1.4) | <5 | -* |
| Pancreatitis | 10 (1.2) | 0 | 10 (2.2) |
| Upper gastrointestinal (GI) | | | |
| Gastro-oesophageal reflux disease (GORD) | 207 (25.8) | 7 (2.7) | 200 (43.6) |
| Peptic ulcer | 0 | 0 | 0 |
| GI bleed (varices as source) | <5 | 0 | <5 |
| GI bleed (non varices as source) | <5 | 0 | <5 |
| Lower gastrointestinal | | | |
| Intestinal obstruction | <5 | <5 | 0 |
| DIOS | 53 (6.6) | <5 | -* |
| Fibrosing colonopathy / colonic stricture | 0 | 0 | 0 |
| Rectal prolapse | 0 | 0 | 0 |
| Renal | | | |
| Kidney stones | <5 | <5 | <5 |
| Renal failure | 6 (0.7) | 0 | 6 (1.3) |
| Musculoskeletal | | | |
| Arthritis | 8 (1.0) | <5 | -* |
| Arthropathy | 24 (3.0) | <5 | -* |
| Bone fracture | <5 | 0 | <5 |
| Osteopenia | 92 (11.5) | <5 | -* |
| Osteoporosis | 41 (5.1) | 0 | 41 (8.9) |
| Other | | | |
| Cancer confirmed by histology | <5 | 0 | <5 |
| Port inserted or replaced | 9 (1.1) | <5 | -* |
| Depression | 9 (1.1) | 0 | 9 (2.0) |
| Hearing loss | 9 (1.1) | <5 | -* |
| Hypertension | 17 (2.1) | 0 | 17 (3.7) |
| Urinary incontinence | 15 (1.9) | <5 | -* |
| Faecal incontinence | 6 (0.7) | <5 | -* |
| Postural anomaly | 13 (1.6) | <5 | -* |

* Redacted to adhere to statistical disclosure guidelines.

1.22 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.

| | 2023 | | |
|---|--------------------|----------------------|----------------------|
| | Overall (n=723) | <16 years (n=264) | ≥16 years (n=459) |
| ABPA; n (%) | 5 (0.7) | 0 | 5 (1.1) |
| Cirrhosis - no portal hypertension; n (%) | <5 | 0 | <5 |
| Cirrhosis - with portal hypertension; n (%) | 6 (0.8) | <5 | <5 |
| Cancer confirmed by histology; n (%) | <5 | 0 | <5 |

1.23 CF diabetes** N=581

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 (n=581) | 10-15 years (n=122) | ≥16 years (n=459) |
|---|--------------------------|------------------------|----------------------|
| On CFD treatment; n(%) | | | |
| Of those on treatment | 95 (16.4) | 12 (9.8) | 83 (18.1) |
| Insulin ¹ ; n(%) | 90 (94.7) | 11 (91.7) | 79 (95.2) |
| CFD Screening; n(%) | | | |
| Yes | 283 (48.7) | 75 (61.5) | 208 (45.3) |
| Screening type; n(%) | | | |
| Continuous glucose monitoring ² ; n(%) | 81 (28.6) | 22 (29.3) | 59 (28.4) |
| Oral glucose tolerance test ² ; n(%) | 238 (84.1) | 51 (68.0) | 187 (89.9) |
| Not screened (other) | 125 (21.5) | <5 | -* |
| Not screened (known CFRD) | 165 (28.4) | 39 (32.0) | 126 (27.5) |
| Unknown | 8 (1.4) | -* | <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

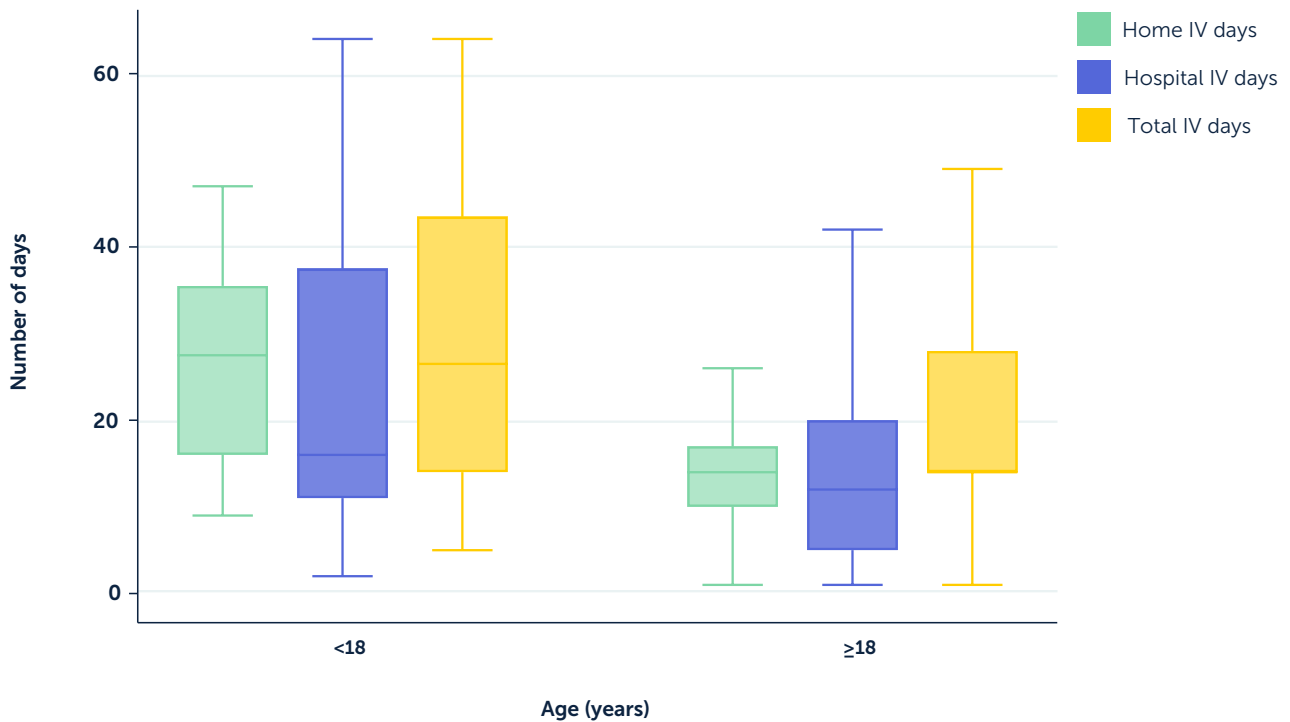
1.24 Intravenous (IV) antibiotics N=723

When someone with CF becomes unwell with an infection, they might be prescribed IV antibiotics. IV antibiotics are given to the patient through their veins. This treatment can take a number of days and might take place as a hospital inpatient, or at home.

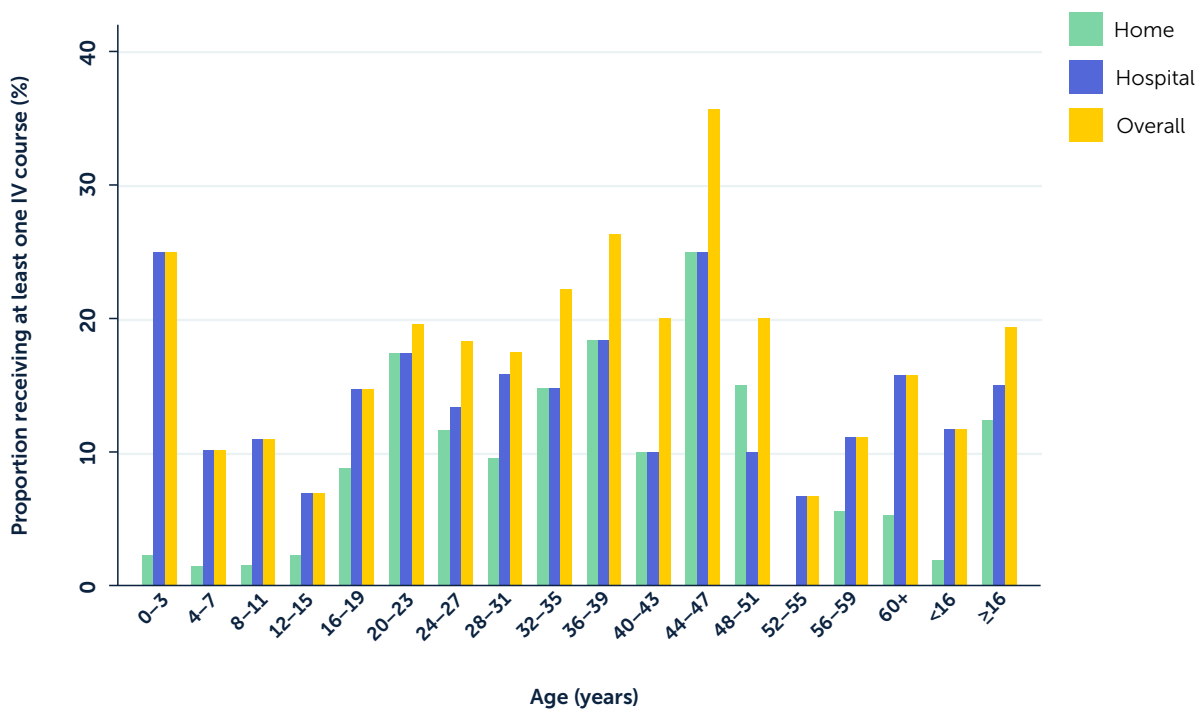
| Age | n | Home | | Hospital | | Total | |
|----------------|------------|-----------------|--------------------|-------------------|--------------------|-------------------|--------------------|
| | | Patients n(%) | Median days (IQR)* | Patients n(%) | Median days (IQR)* | Patients n (%) | Median days (IQR)* |
| 0-3 | 44 | <5 | * | 11 (25.0) | 15 (14-38) | 11 (25.0) | 15 (14-39) |
| 4-7 | 69 | <5 | * | 7 (10.1) | 17 (7-64) | 7 (10.1) | 26 (14-64) |
| 8-11 | 64 | <5 | * | 7 (10.9) | 16 (13-37) | 7 (10.9) | 21 (13-56) |
| 12-15 | 87 | <5 | * | 6 (6.9) | 20 (9-32) | 6 (6.9) | 30 (27-41) |
| 16-19 | 68 | 6 (8.8) | 12 (10-36) | 10 (14.7) | 10 (3-21) | 10 (14.7) | 18 (14-52) |
| 20-23 | 46 | 8 (17.4) | 11 (6-19) | 8 (17.4) | 10 (7-16) | 9 (19.6) | 21 (14-28) |
| 24-27 | 60 | 7 (11.7) | 14 (7-19) | 8 (13.3) | 11 (6-24) | 11 (18.3) | 14 (14-41) |
| 28-31 | 63 | 6 (9.5) | 18 (13-23) | 10 (15.9) | 6 (4-35) | 11 (17.5) | 28 (14-40) |
| 32-35 | 54 | 8 (14.8) | 14 (12-14) | 8 (14.8) | 14 (8-15) | 12 (22.2) | 14 (14-22) |
| 36-39 | 38 | 7 (18.4) | 13 (10-14) | 7 (18.4) | 6 (1-28) | 10 (26.3) | 14 (14-19) |
| 40-43 | 30 | <5 | * | <5 | * | 6 (20.0) | 14 (14-14) |
| 44-47 | 28 | 7 (25.0) | 14 (4-42) | 7 (25.0) | 13 (3-42) | 10 (35.7) | 14 (13-56) |
| 48-51 | 20 | <5 | * | <5 | * | <5 | * |
| 52-55 | 15 | 0 (0.0) | n/a | <5 | * | <5 | * |
| 56-59 | 18 | <5 | * | <5 | * | <5 | * |
| 60+ | 19 | <5 | * | <5 | * | <5 | * |
| <16 | 264 | 5 (1.9) | 29 (26-35) | 31 (11.7) | 16 (12-37) | 31 (11.7) | 26 (14-42) |
| ≥16 | 459 | 57 (12.4) | 14 (10-17) | 69 (15.0) | 12 (5-21) | 89 (19.4) | 14 (14-28) |
| <18 | 293 | 8 (2.7) | 28 (16-36) | 36 (12.3) | 16 (11-38) | 36 (12.3) | 26 (14-44) |
| ≥18 | 430 | 54 (12.6) | 14 (10-17) | 64 (14.9) | 12 (5-20) | 84 (19.5) | 14 (14-28) |
| Overall | 723 | 62 (8.6) | 14 (10-22) | 100 (13.8) | 14 (6-28) | 120 (16.6) | 14 (14-38) |

* 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 51.



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 13.8%. The proportion receiving any IVs was 16.6%.



1.25 Inhaled antibiotic use N=723

| | 2023 | | |
|--|-------------------|------------------|-------------------|
| | Overall | <16 years | ≥16 years |
| Number of patients | 723 | 264 | 459 |
| Tobramycin solution; n(%) | 35 (4.8) | 13 (4.9) | 22 (4.8) |
| Other aminoglycoside; n(%) | <5 | 0 | <5 |
| Colistin; n(%) | 47 (6.5) | 21 (8.0) | 26 (5.7) |
| Promixin; n(%) | 25 (3.5) | 5 (1.9) | 20 (4.4) |
| Aztreonam; n(%) | 12 (1.7) | 0 | 12 (2.6) |
| Colistimethate (DPI); n(%) | 30 (4.1) | <5 | -* |
| Tobramycin Inhalation Powder; n(%) | 16 (2.2) | <5 | -* |
| Levofloxacin; n(%) | <5 | 0 | <5 |
| At least one of the above; n(%) | 144 (19.9) | 34 (12.9) | 110 (24.0) |

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

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

| | 2023 | | |
|---|------------------|--------------|------------------|
| | Overall | <16 years | ≥16 years |
| Patients with chronic <i>P. aeruginosa</i> | 35 | 3 | 32 |
| Tobramycin solution; n(%) | <5 | <5 | <5 |
| Other aminoglycoside; n(%) | 0 | 0 | 0 |
| Colistin; n(%) | 10 (28.6) | <5 | -* |
| Promixin; n(%) | <5 | 0 | <5 |
| Aztreonam; n(%) | <5 | 0 | <5 |
| Colistimethate (DPI); n(%) | <5 | 0 | <5 |
| Tobramycin Inhalation Powder; n(%) | <5 | 0 | <5 |
| Levofloxacin; n(%) | <5 | 0 | <5 |
| At least one of the above; n(%) | 23 (65.7) | <5 | 20 (62.5) |

* Redacted to adhere to statistical disclosure guidelines.

1.27 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* infection.

| | | Number of patients on azithromycin; n | Patients with chronic <i>P. aeruginosa</i> ; n(%) | Patients without chronic <i>P. aeruginosa</i> ; n(%) |
|------|------------|---------------------------------------|---|--|
| 2013 | 0-3 years | <5 | <5 | <5 |
| | 4-15 years | 59 | 8 (13.6) | 51 (86.4) |
| | ≥ 16 years | 326 | 176 (54.0) | 150 (46.0) |
| 2018 | 0-3 years | <5 | 0 | <5 |
| | 4-15 years | 69 | 10 (14.5) | 59 (85.5) |
| | ≥ 16 years | 343 | 161 (46.9) | 182 (53.1) |
| 2023 | 0-3 years | -* | <5 | <5 |
| | 4-15 years | -* | <5 | 55 (98.2) |
| | ≥ 16 years | 224 | 29 (12.9) | 195 (87.1) |

1.28 Flucloxacillin use

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

| Age | 2018 | | 2023 | |
|----------------|----------------|---|----------------|---|
| | Total patients | Patients on prophylactic flucloxacillin; n(%) | Total patients | Patients on prophylactic flucloxacillin; n(%) |
| 0-3 | 55 | 35 (63.6) | 44 | 23 (52.3) |
| 4-7 | 82 | 27 (32.9) | 69 | 21 (30.4) |
| 8-11 | 75 | 21 (28.0) | 64 | 18 (28.1) |
| 12-15 | 92 | 31 (33.7) | 87 | 12 (13.8) |
| 16-19 | 53 | 11 (20.8) | 68 | <5 |
| 20-23 | 91 | 22 (24.2) | 46 | <5 |
| 24-27 | 82 | 16 (19.5) | 60 | 11 (18.3) |
| 28-31 | 65 | 8 (12.3) | 63 | 6 (9.5) |
| 32-35 | 64 | 5 (7.8) | 54 | <5 |
| 36-39 | 41 | <5 | 38 | <5 |
| 40-43 | 31 | 0 | 30 | 0 |
| 44-47 | 18 | 0 | 28 | 0 |
| 48-51 | 26 | <5 | 20 | 0 |
| 52-55 | 23 | <5 | 15 | <5 |
| 56-59 | 8 | 0 | 18 | <5 |
| 60+ | 13 | 0 | 19 | 0 |
| <16 years | 304 | 114 (37.5) | 264 | 74 (28.0) |
| ≥16 years | 515 | 69 (13.4) | 459 | 31 (6.8) |
| <18 years | 325 | 116 (35.7) | 293 | 76 (25.9) |
| ≥18 years | 494 | 67 (13.6) | 430 | 29 (6.7) |
| Overall | 819 | 183 (22.3) | 723 | 105 (14.5) |

* Redacted to adhere to statistical disclosure guidelines.

1.29 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 | 73 | 6 (8.2) | <5 | <5 |
| 6 - ≤16 years | 204 | 42 (20.6) | 23 (11.3) | 11 (5.4) |
| 6 - ≤18 years | 242 | 58 (24.0) | 29 (12.0) | 13 (5.4) |
| <16 years | 264 | 42 (15.9) | 27 (10.2) | 11 (4.2) |
| ≥16 years | 459 | 290 (63.2) | 98 (21.4) | 85 (18.5) |
| <18 years | 293 | 54 (18.4) | 29 (9.9) | 14 (4.8) |
| ≥18 years | 430 | 278 (64.7) | 96 (22.3) | 82 (19.1) |
| Overall | 723 | 332 (45.9) | 125 (17.3) | 96 (13.3) |

Mucoactive therapies

1.30 Mannitol

| Age | 2018 | | 2023 | |
|----------------|----------------|----------------------------|----------------|----------------------------|
| | Total patients | Patients on Mannitol; n(%) | Total patients | Patients on Mannitol; n(%) |
| 0-3 | 55 | 0 | 44 | 0 |
| 4-7 | 82 | 0 | 69 | 0 |
| 8-11 | 75 | 0 | 64 | 0 |
| 12-15 | 92 | 0 | 87 | 0 |
| 16-19 | 53 | 0 | 68 | 0 |
| 20-23 | 91 | 0 | 46 | 0 |
| 24-27 | 82 | 0 | 60 | 0 |
| 28-31 | 65 | <5 | 63 | 0 |
| 32-35 | 64 | <5 | 54 | 0 |
| 36-39 | 41 | <5 | 38 | <5 |
| 40-43 | 31 | <5 | 30 | 0 |
| 44-47 | 18 | 0 | 28 | 0 |
| 48-51 | 26 | <5 | 20 | <5 |
| 52-55 | 23 | 0 | 15 | 0 |
| 56-59 | 8 | 0 | 18 | 0 |
| 60+ | 13 | 0 | 19 | 0 |
| <16 years | 304 | 0 | 264 | 0 |
| ≥16 years | 515 | 9 (1.7) | 459 | <5 |
| <18 years | 325 | 0 | 293 | 0 |
| ≥18 years | 494 | 9 (1.8) | 430 | <5 |
| Overall | 819 | 9 (1.1) | 723 | <5 |

1.31 DNase

| Age | 2013 | | 2018 | | 2023 | |
|----------------|----------------|-------------------------|----------------|-------------------------|----------------|-------------------------|
| | Total patients | Patients on DNase; n(%) | Total patients | Patients on DNase; n(%) | Total patients | Patients on DNase; n(%) |
| 0-3 | 70 | <5 | 55 | <5 | 44 | 6 (13.6) |
| 4-7 | 101 | 11 (10.9) | 82 | 22 (26.8) | 69 | 22 (31.9) |
| 8-11 | 82 | 22 (26.8) | 75 | 32 (42.7) | 64 | 29 (45.3) |
| 12-15 | 61 | 27 (44.3) | 92 | 51 (55.4) | 87 | 40 (46.0) |
| 16-19 | 99 | 41 (41.4) | 53 | 24 (45.3) | 68 | 39 (57.4) |
| 20-23 | 97 | 42 (43.3) | 91 | 57 (62.6) | 46 | 18 (39.1) |
| 24-27 | 75 | 29 (38.7) | 82 | 39 (47.6) | 60 | 28 (46.7) |
| 28-31 | 67 | 25 (37.3) | 65 | 42 (64.6) | 63 | 31 (49.2) |
| 32-35 | 49 | 12 (24.5) | 64 | 26 (40.6) | 54 | 27 (50.0) |
| 36-39 | 26 | 7 (26.9) | 41 | 13 (31.7) | 38 | 14 (36.8) |
| 40-43 | 19 | <5 | 31 | 14 (45.2) | 30 | 10 (33.3) |
| 44-47 | 22 | 7 (31.8) | 18 | 7 (38.9) | 28 | 11 (39.3) |
| 48-51 | 20 | 6 (30.0) | 26 | 12 (46.2) | 20 | 7 (35.0) |
| 52-55 | 7 | <5 | 23 | 8 (34.8) | 15 | <5 |
| 56-59 | 7 | 0 | 8 | <5 | 18 | 8 (44.4) |
| 60+ | 7 | <5 | 13 | 5 (38.5) | 19 | <5 |
| <16 years | 314 | 63 (20.1) | 304 | 106 (34.9) | 264 | 97 (36.7) |
| ≥16 years | 496 | 179 (36.1) | 515 | 249 (48.3) | 459 | 199 (43.4) |
| <18 years | 363 | 86 (23.7) | 325 | 115 (35.4) | 293 | 118 (40.3) |
| ≥18 years | 447 | 156 (34.9) | 494 | 240 (48.6) | 430 | 178 (41.4) |
| Overall | 810 | 242 (29.9) | 819 | 355 (43.3) | 723 | 296 (40.9) |

1.32 Hypertonic saline

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

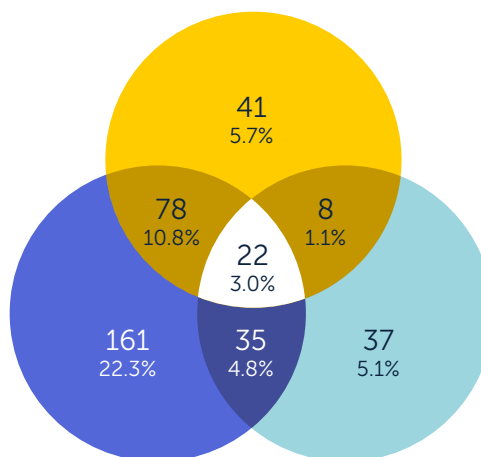
| Age | 2013 | | 2018 | | 2023 | |
|----------------|----------------|-------------------------------------|----------------|-------------------------------------|----------------|-------------------------------------|
| | Total patients | Patients on hypertonic saline; n(%) | Total patients | Patients on hypertonic saline; n(%) | Total patients | Patients on hypertonic saline; n(%) |
| 0-3 | 70 | <5 | 55 | 7 (12.7) | 44 | <5 |
| 4-7 | 101 | 6 (5.9) | 82 | 8 (9.8) | 69 | 18 (26.1) |
| 8-11 | 82 | 10 (12.2) | 75 | 17 (22.7) | 64 | 17 (26.6) |
| 12-15 | 61 | 12 (19.7) | 92 | 32 (34.8) | 87 | 20 (23.0) |
| 16-19 | 99 | 26 (26.3) | 53 | 16 (30.2) | 68 | 10 (14.7) |
| 20-23 | 97 | 25 (25.8) | 91 | 26 (28.6) | 46 | 5 (10.9) |
| 24-27 | 75 | 9 (12.0) | 82 | 16 (19.5) | 60 | 7 (11.7) |
| 28-31 | 67 | 7 (10.4) | 65 | 10 (15.4) | 63 | 6 (9.5) |
| 32-35 | 49 | 5 (10.2) | 64 | 13 (20.3) | 54 | 6 (11.1) |
| 36-39 | 26 | 0 | 41 | 5 (12.2) | 38 | <5 |
| 40-43 | 19 | <5 | 31 | <5 | 30 | <5 |
| 44-47 | 22 | <5 | 18 | <5 | 28 | 0 |
| 48-51 | 20 | <5 | 26 | <5 | 20 | <5 |
| 52-55 | 7 | <5 | 23 | <5 | 15 | 0 |
| 56-59 | 7 | 0 | 8 | <5 | 18 | 0 |
| 60+ | 7 | <5 | 13 | 0 | 19 | <5 |
| <16 years | 314 | 29 (9.2) | 304 | 64 (21.1) | 264 | 58 (22.0) |
| ≥16 years | 496 | 81 (16.3) | 515 | 96 (18.6) | 459 | 42 (9.2) |
| <18 years | 363 | 46 (12.7) | 325 | 72 (22.2) | 293 | 66 (22.5) |
| ≥18 years | 447 | 64 (14.3) | 494 | 88 (17.8) | 430 | 34 (7.9) |
| Overall | 810 | 110 (13.6) | 819 | 160 (19.5) | 723 | 100 (13.8) |

1.33 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 382 (52.8%) people in Scotland are on no inhaled therapies.

**None of these inhaled medications:
382 (52.8%)**

- Inhaled antibiotics
- DNase
- Hypertonic saline or mannitol



CFTR modulators

During 2023 the CFTR modulators (CFTRm) were made available to people with cystic fibrosis under a managed access agreement.

Ivacaftor

In 2023 ivacaftor had approval for use in people aged 4 months and older with at least one copy of a CFTR “gating” variant and for people with the R117H variant.

Lumacaftor/ivacaftor

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

Tezacaftor/ivacaftor

Tezacaftor / ivacaftor is licensed for use in people with CF aged six years and over who have two copies of the F508del variant, or a single copy of F508del and one of 14 “residual” function variants.

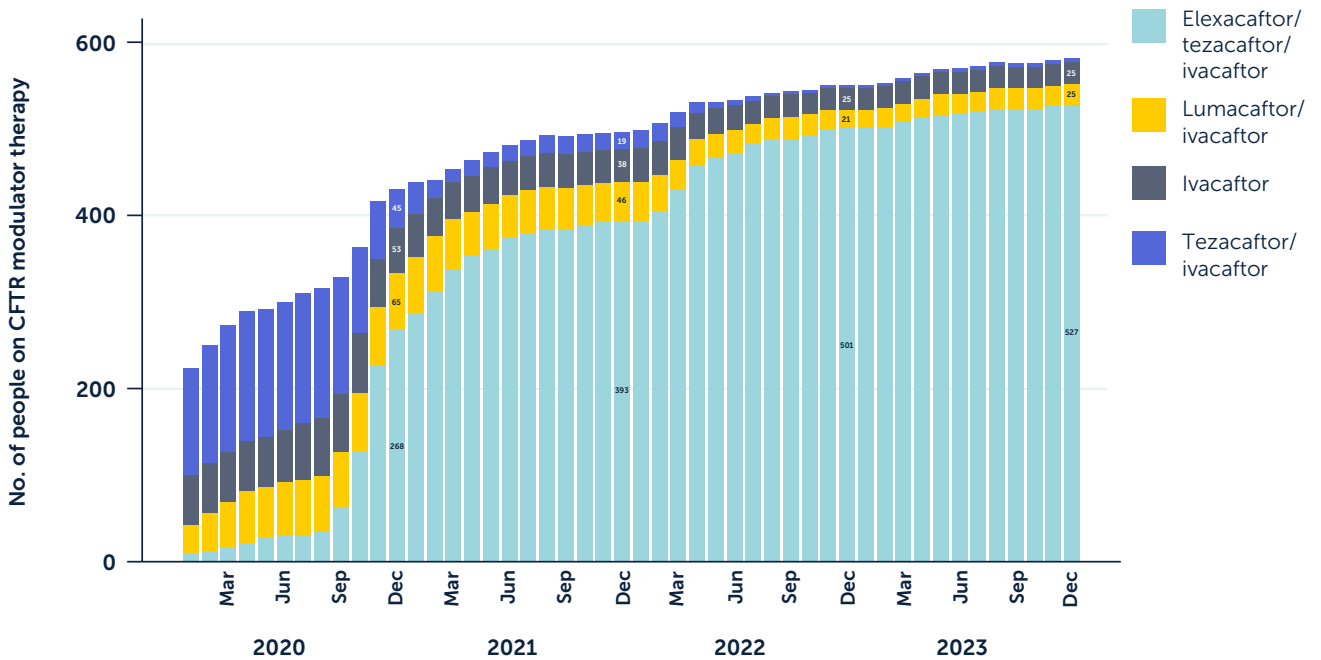
Elexacaftor/tezacaftor/ivacaftor

Elexacaftor/ tezacaftor/ ivacaftor was available for people with CF aged six years and over who have two copies of the F508del variant, or a single copy of F508del and one minimal function variant. In November 2023, this was extended to include children aged two and over. NHS commissioning statements adopted across the UK support the prescribing of CFTR modulators “off label”; the arrangement varies slightly across devolved nations but covers the 177 CFTR variants on an approved “FDA list”, and in some devolved nations, responsive genotypes identified through the “French Compassionate Use Programme”.

Access arrangements for the CFTR modulators prior to 2023 can be found in previous annual reports and on our website here: cysticfibrosis.org.uk/treatmentsandmedication.

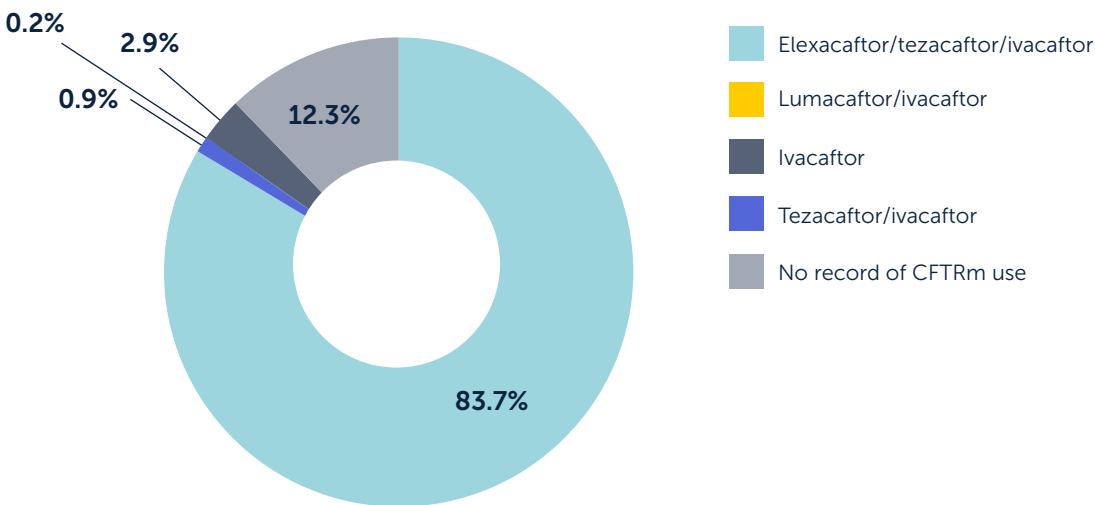
1.34 CFTR modulator use in 2023

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. By December, 581 people were taking a CFTR modulator in Scotland.



1.35a CFTR modulator use in all people aged six years and older¹ N=657

The chart shows the distribution of CFTR modulators taken in those aged 6 and over as of 31/12/2023. The last CFTR modulator as of 31/12/2023 is shown in the chart. 15.6% of people aged 6 and over had no record of any CFTRm use on the Registry.



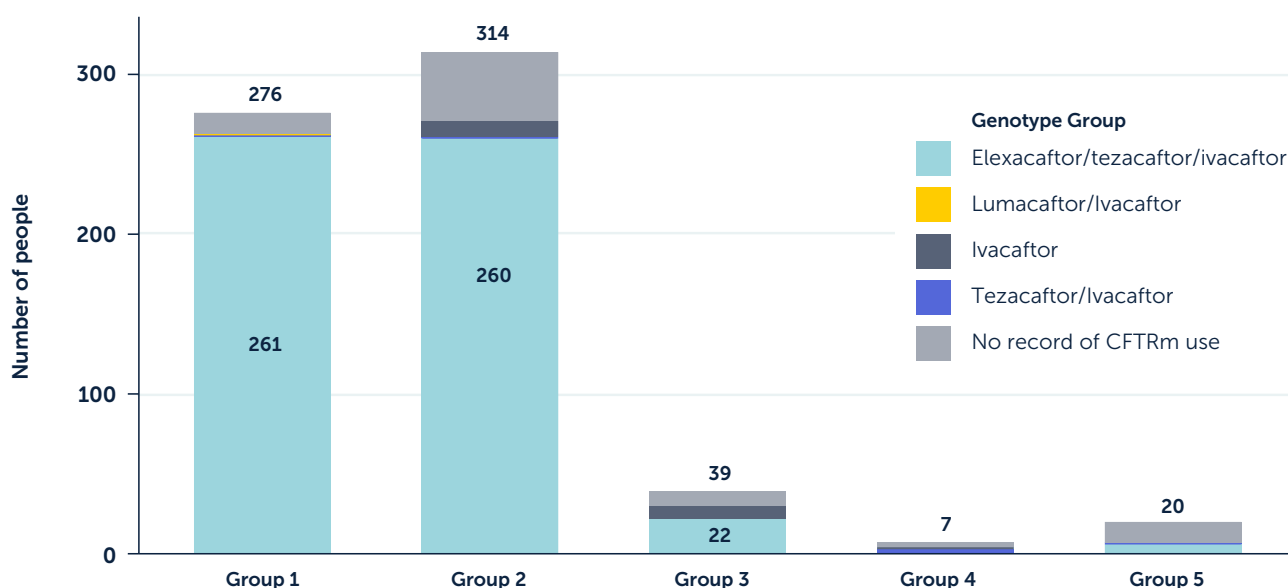
¹ Fewer than 5 people excluded because their last recorded CFTRm treatment was a part of a drug trial where the specific drug was unknown.

1.35b CFTR modulator use in all people aged six years and older by genotype group^{1,2}

N=657

The chart below shows the most recent CFTR modulator recorded for people aged six and older and according to their genotype group as defined in the table below. The full list of CFTR variants included within these groups can be found in appendix 3.

Some people with a potentially responsive variant (Groups 1 to 4) have no recorded use of any CFTR modulator on the Registry. There could be several reasons for this: for example, a person may have had a transplant or there could be a data entry error. There are a small number of people³ thought to have non-responsive genotypes (group 5) who are currently taking or have previously taken a modulator. It is not possible to tell from the Registry record why they received a CFTR modulator.



| Genotype Group Definitions | |
|----------------------------|--|
| Group 1 | F508del Homozygous |
| Group 2 | F508del Heterozygous |
| Group 3 | no F508del, but ETI* responsive variant as defined by FDA list (3a**) or French Compassionate Use Programme list (3b**) |
| Group 4 | no F508del, no ETI* responsive variant as defined by FDA list (3a**) or French Compassionate Use Programme list (3b**), but at least 1 variant from lists 3c** or 3d** |
| Group 5 | no F508del, no ETI* responsive variant as defined by FDA list (3a**) or French Compassionate Use Programme list (3b**), no variant from lists 3c** or 3d** |

¹ Fewer than five patients were excluded because their last recorded CFTRm treatment was as part of a drug trial and the specific drug was unknown

² Age as of 31/12/2023

³ For number and % details see Appendix 3 Table 3e

* ETI is Elexacaftor/tezacaftor/ivacaftor

** see Appendix 3

1.36 Demographic characteristics for people aged six years and older, by genotype group and CFTR modulator use^{1,2}

| | All potential responders [*] | | Likely non-responders ^{**4} |
|---------------------------|---------------------------------------|-------------------------------------|--------------------------------------|
| | CFTRm use recorded ³ | No record of CFTRm use ³ | No record of CFTRm use ³ |
| Number of individuals (n) | 568 | 68 | 13 |
| Male n (%) | 311 (54.8) | 31 (45.6) | 7 (53.9) |
| Ethnicity | | | |
| White n (%) | 559 (98.4) | 63 (92.7) | 7 (53.9) |
| Asian n (%) | -*** | <5 | -*** |
| Black n (%) | 0 | 0 | <5 |
| Mixed n (%) | <5 | <5 | 0 |
| Other n (%) | 0 | 0 | 0 |
| Age (years) | | | |
| Mean (sd) | 27 (15) | 33 (17) | 23 (11) |
| Median (IQR) | 25 (15, 36) | 30 (16, 47) | 20 (16, 31) |

¹ Fewer than five patients were excluded because their last recorded CFTRm treatment was as part of a drug trial and the specific drug was unknown

² Age as of 31/12/2023

³ "CFTRm record/no record of CFTRm use" defined as if a patient had any CFTRm record as of 31/12/2023.

⁴ Details for seven people considered non-responders who had a record of CFTRm use are not included

* Defined at least one F508del or ETI responsive variant as defined by FDA list (3a) or French Compassionate Use list (3b) or variant from lists 3c or 3d.

** Defined as no F508del, no ETI responsive variant as defined by FDA list (3a) or French Compassionate Use list (3b), and no variant from lists 3c or 3d.

*** Redacted to adhere to statistical disclosure guidelines.

Other therapies

1.37 Physiotherapy

Physiotherapy helps people with CF clear sticky mucus from their lungs.

| | Overall (n=723) | <16 years (n=264) | ≥16 years (n=459) | <18 years (n=293) | ≥18 years (n=430) |
|--|-----------------|-------------------|-------------------|-------------------|-------------------|
| Active Cycle of Breathing Techniques | 53 (7.3) | 8 (3.0) | 45 (9.8) | 8 (2.7) | 45 (10.5) |
| Assisted autogenic drainage | 80 (11.1) | 5 (1.9) | 75 (16.3) | 9 (3.1) | 71 (16.5) |
| Autogenic drainage | 99 (13.7) | <5 | 98 (21.4) | <5 | 98 (22.8) |
| Exercise; of which: | 72 (10.0) | 7 (2.7) | 65 (14.2) | 10 (3.4) | 62 (14.4) |
| Exercise listed as only airway clearance technique | 48 (6.6) | 5 (1.9) | 43 (9.4) | 7 (2.4) | 41 (9.5) |
| Forced expiration | 12 (1.7) | 0 (0.0) | 12 (2.6) | 0 (0.0) | 12 (2.8) |
| High Pressure PEP | 14 (1.9) | 12 (4.5) | <5 | 12 (4.1) | <5 |
| Manual techniques (percussion over pressures vibrations) | <5 | <5 | <5 | <5 | <5 |
| Oscillating PEP | 54 (7.5) | 37 (14.0) | 17 (3.7) | 43 (14.7) | 11 (2.6) |
| PEP | 269 (37.2) | 182 (68.9) | 87 (19.0) | 195 (66.6) | 74 (17.2) |
| Postural drainage | <5 | 0 (0.0) | <5 | 0 (0.0) | <5 |
| VEST | <5 | 0 (0.0) | <5 | 0 (0.0) | <5 |
| Other | 12 (1.7) | <5 | 9 (2.0) | <5 | 8 (1.9) |
| None | 53 (7.3) | 8 (3.0) | 45 (9.8) | 10 (3.4) | 43 (10.0) |

Note that these techniques are not mutually exclusive and represent primary and secondary forms of physiotherapy.

1.38 Primary or secondary airway clearance technique

| | Overall (n=723) | <16 years (n=264) | ≥16 years (n=459) | <18 years (n=293) | ≥18 years (n=430) |
|--|-----------------|-------------------|-------------------|-------------------|-------------------|
| Active cycle of breathing techniques | 79 (10.9) | 12 (4.5) | 67 (14.6) | 13 (4.4) | 66 (15.3) |
| Assisted autogenic drainage | 108 (14.9) | 21 (8.0) | 87 (19.0) | 25 (8.5) | 83 (19.3) |
| Autogenic drainage | 171 (23.7) | 23 (8.7) | 148 (32.2) | 26 (8.9) | 145 (33.7) |
| Exercise | 428 (59.2) | 122 (46.2) | 306 (66.7) | 146 (49.8) | 282 (65.6) |
| Forced expiration | 42 (5.8) | 7 (2.7) | 35 (7.6) | 9 (3.1) | 33 (7.7) |
| High pressure PEP | 14 (1.9) | 12 (4.5) | <5 | 12 (4.1) | <5 |
| Manual techniques (percussion over pressures vibrations) | 6 (0.8) | <5 | <5 | <5 | <5 |
| Oscillating PEP | 77 (10.7) | 43 (16.3) | 34 (7.4) | 52 (17.7) | 25 (5.8) |
| PEP | 309 (42.7) | 196 (74.2) | 113 (24.6) | 210 (71.7) | 99 (23.0) |
| Postural drainage | <5 | <5 | <5 | <5 | <5 |
| VEST | <5 | 0 (0.0) | <5 | 0 (0.0) | <5 |
| Other | 110 (15.2) | 87 (33.0) | 23 (5.0) | 89 (30.4) | 21 (4.9) |

1.39 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=723) | <16 years (n=264) | ≥16 years (n=459) | <18 years (n=293) | ≥18 years (n=430) |
|--------------------------------------|--------------------|----------------------|----------------------|----------------------|----------------------|
| Yes* | 153 (17.3) | 25 (8.5) | 128 (21.6) | 32 (9.7) | 121 (21.8) |
| No | 426 (48.1) | 155 (52.7) | 271 (45.8) | 169 (51.2) | 257 (46.2) |
| Not known or missing | 144 (16.3) | 84 (28.6) | 60 (10.1) | 92 (27.9) | 52 (9.4) |
| Type of exercise test ^{1,2} | | | | | |
| CPET | 17 (11.1) | 17 (68.0) | 0 (0.0) | 17 (53.1) | 0 (0.0) |
| Shuttle test | 10 (6.5) | 5 (20.0) | 5 (3.9) | 5 (15.6) | 5 (4.1) |
| Step test | 38 (24.8) | 0 (0.0) | 38 (29.7) | <5 | 37 (30.6) |
| 6 minute walk test | 8 (5.2) | <5 | <5 | <5 | <5 |
| Other | 12 (7.8) | 0 (0.0) | 12 (9.4) | <5 | -* |
| Missing | 78 (51.0) | <5 | 74 (57.8) | -* | 69 (57.0) |

* Exercise test represents all types of testing listed including Cardiopulmonary Exercise Test (CPET), shuttle test, 6 minute walk test, step test and other test.

¹ Proportion of patients who answered Yes above.

² More than one type of test can be recorded so % total may not sum to 100%.

1.40 Oxygen and non-invasive ventilation

| | Overall (n=723) | <16 years (n=264) | ≥16 years (n=459) | <18 years (n=293) | ≥18 years (n=430) |
|--|--------------------|----------------------|----------------------|----------------------|----------------------|
| Non invasive ventilation (NIV); n (%) | <5 | 0 (0.0) | <5 | 0 (0.0) | <5 |
| Any oxygen use; n (%) | 23 (3.2) | <5 | -* | <5 | -* |
| Among those who had oxygen use: | | | | | |
| Continuously | <5 | 0 (0.0) | <5 | 0 (0.0) | <5 |
| Nocturnal or with exertion | 9 (39.1) | 0 (0.0) | 9 (47.4) | 0 (0.0) | 9 (47.4) |
| As required (PRN) | <5 | 0 (0.0) | <5 | 0 (0.0) | <5 |
| With exacerbation | 6 (26.1) | <5 | <5 | <5 | <5 |

1.41 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 over time.

| | 2018 | 2019 | 2020 | 2021 | 2022 | 2023 |
|--|------|------|------|------|------|------|
| Patients evaluated; n | 19 | 21 | 11 | 5 | 10 | 3 |
| Patients accepted; n | 7 | 10 | <5 | <5 | <5 | <5 |
| Patients receiving transplants; n | <5 | 5 | 0 | <5 | <5 | <5 |
| Bilateral lung | <5 | <5 | 0 | <5 | 0 | 0 |
| Liver | <5 | <5 | 0 | 0 | 0 | 0 |
| Other | <5 | 0 | 0 | 0 | <5 | <5 |

1.42 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.

| Year | | Overall | <16 years | ≥16 years | <18 years | ≥18 years |
|-------------|--|------------|------------|------------|------------|------------|
| 2013 | Total; n | 810 | 314 | 496 | 363 | 447 |
| 2013 | Any supplemental feeding; n(%) | 199 (24.6) | 64 (20.4) | 135 (27.2) | 78 (21.5) | 121 (27.1) |
| 2013 | Oral; n(%) | 155 (19.1) | 50 (15.9) | 105 (21.2) | 59 (16.3) | 96 (21.5) |
| 2013 | Nasogastric tube; n(%) | 9 (1.1) | 0 (0.0) | 9 (1.8) | <5 | 7 (1.6) |
| 2013 | Gastrostomy tube/Button; n(%) | 46 (5.7) | 15 (4.8) | 31 (6.2) | 20 (5.5) | 26 (5.8) |
| 2013 | Jejunal; n(%) | 0 (0.0) | 0 (0.0) | 0 (0.0) | 0 (0.0) | 0 (0.0) |
| 2013 | Total Parenteral Nutrition (TPN); n(%) | 0 (0.0) | 0 (0.0) | 0 (0.0) | 0 (0.0) | 0 (0.0) |
| 2018 | Total; n | 819 | 304 | 515 | 325 | 494 |
| 2018 | Any supplemental feeding; n(%) | 191 (23.3) | 62 (20.4) | 129 (25.0) | 65 (20.0) | 126 (25.5) |
| 2018 | Oral; n(%) | 140 (17.1) | 39 (12.8) | 101 (19.6) | 42 (12.9) | 98 (19.8) |
| 2018 | Nasogastric tube; n(%) | 11 (1.3) | <5 | -* | <5 | -* |
| 2018 | Gastrostomy tube/Button; n(%) | 33 (4.0) | 15 (4.9) | 18 (3.5) | 15 (4.6) | 18 (3.6) |
| 2018 | Jejunal; n(%) | 0 (0.0) | 0 (0.0) | 0 (0.0) | 0 (0.0) | 0 (0.0) |
| 2018 | Total Parenteral Nutrition (TPN); n(%) | 0 (0.0) | 0 (0.0) | 0 (0.0) | 0 (0.0) | 0 (0.0) |
| 2023 | Total; n | 723 | 264 | 459 | 293 | 430 |
| 2023 | Any supplemental feeding; n(%) | 123 (17.0) | 39 (14.8) | 84 (18.3) | 45 (15.4) | 78 (18.1) |
| 2023 | Oral; n(%) | 72 (10.0) | 21 (8.0) | 51 (11.1) | 23 (7.8) | 49 (11.4) |
| 2023 | Nasogastric tube; n(%) | <5 | <5 | <5 | <5 | 0 (0.0) |
| 2023 | Gastrostomy tube/Button; n(%) | 14 (1.9) | 7 (2.7) | 7 (1.5) | 9 (3.1) | 5 (1.2) |
| 2023 | Jejunal; n(%) | 0 (0.0) | 0 (0.0) | 0 (0.0) | 0 (0.0) | 0 (0.0) |
| 2023 | Total Parenteral Nutrition (TPN); n(%) | 0 (0.0) | 0 (0.0) | 0 (0.0) | 0 (0.0) | 0 (0.0) |

1.43 Pancreatic enzyme supplementation

| Year | | Overall | <16 years | ≥16 years | <18 years | ≥18 years |
|-------------|-------------------------------------|------------|------------|------------|------------|------------|
| 2013 | Total; n | 810 | 314 | 496 | 363 | 447 |
| 2013 | Pancreatic enzyme supplements; n(%) | 676 (83.5) | 267 (85.0) | 409 (82.5) | 311 (85.7) | 365 (81.7) |
| 2018 | Total; n | 819 | 304 | 515 | 325 | 494 |
| 2018 | Pancreatic enzyme supplements; n(%) | 650 (79.4) | 233 (76.6) | 417 (81.0) | 250 (76.9) | 400 (81.0) |
| 2023 | Total; n | 723 | 264 | 459 | 293 | 430 |
| 2023 | Pancreatic enzyme supplements; n(%) | 555 (76.8) | 198 (75.0) | 357 (77.8) | 222 (75.8) | 333 (77.4) |

* 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 variant recorded | 968 (99.6) |
| Patients genotyped with both variants recorded | 960 (98.8) |
| F508del mutations | |
| Homozygous F508del | 406 (41.8) |
| Heterozygous F508del | 470 (48.4) |

1.44 Variant combinations in Scotland

This table shows the proportion (%) of patients with the most common mutation combinations. For example, 7.5% of the Scottish population have one copy of F508del and one copy of G551D in their genotype.

| Mutation 2 | Mutation 1 | | | | | | | Total |
|--------------|-------------|------------|------------|------------|------------|------------|------------|------------|
| | F508del | R117H | G551D | G542X | 621+1G->T | Other | Unknown | |
| | (%) | | | | | | | |
| F508del | 41.8 | | | | | | | 41.8 |
| R117H | 6.7 | 0.1 | | | | | | 6.8 |
| G551D | 7.5 | 0.1 | 0.2 | | | | | 7.8 |
| G542X | 5.0 | 0.2 | 0.1 | 0.1 | | | | 5.5 |
| 621+1G->T | 0.6 | 0.0 | 0.1 | 0.0 | 0.0 | | | 0.7 |
| Other | 27.9 | 0.9 | 1.6 | 0.9 | 0.3 | 4.5 | | 36.2 |
| Unknown | 0.6 | 0.1 | 0.1 | 0.0 | 0.0 | 0.0 | 0.4 | 1.2 |
| Total | 90.1 | 1.4 | 2.2 | 1.0 | 0.3 | 4.5 | 0.4 | 100 |

* In this section, we include everyone who is registered (see table 1.1) and where mutations are available.

1.45 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 | 876 | 90.1 |
| c.1652G->A | p.Gly551Asp | G551D | 95 | 9.8 |
| c.350G->A | p.Arg117His | R117H | 79 | 8.1 |
| c.1624G->T | p.Gly542X | G542X | 62 | 6.4 |
| c.200C->T | p.Pro67Leu | P67L | 54 | 5.6 |
| c.3454G->C | p.Asp1152His | D1152H | 23 | 2.4 |
| c.1679G->C | p.Arg560Thr | R560T | 18 | 1.9 |
| c.1585-1G->A | | 1717-1G->A | 18 | 1.9 |
| c.1477C->T | p.Gln493X | Q493X | 15 | 1.5 |
| c.2657+5G->A | | 2789+5G->A | 14 | 1.4 |
| c.3909C->G | p.Asn1303Lys | N1303K | 12 | 1.2 |
| c.1135G->T | p.Glu379X | E379X | 12 | 1.2 |
| c.489+1G->T | | 621+1G->T | 10 | 1.0 |
| c.3717+12191C->T | | 3849+10kbC->T | 10 | 1.0 |
| c.1364C->A | p.Ala455Glu | A455E | 9 | 0.9 |
| c.3528delC | p.Lys1177SerfsX15 | 3659delC | 9 | 0.9 |
| c.1558G->T | p.Val520Phe | V520F | 8 | 0.8 |
| c.178G->T | p.Glu60X | E60X | 8 | 0.8 |
| c.3140-26A->G | | 3272-26A->G | 7 | 0.7 |
| c.2657+2_2657+3insA | | 2789+2insA | 7 | 0.7 |

Section 2: Centre-level analysis

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

| | Paediatric | Adult | Total |
|---------------------|------------|-------|-------|
| Centres | 5 | 3 | 8 |
| Stand-alone clinics | 1 | 0 | 1 |

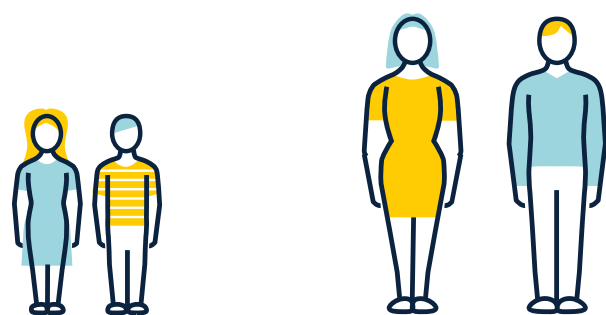
Section 2 shows analysis of data for individual CF centres. This allows people with CF, their families, and healthcare providers to review a centre's use of some medications and outcome data alongside national averages. This transparency is intended to help improve standards of care overall.

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



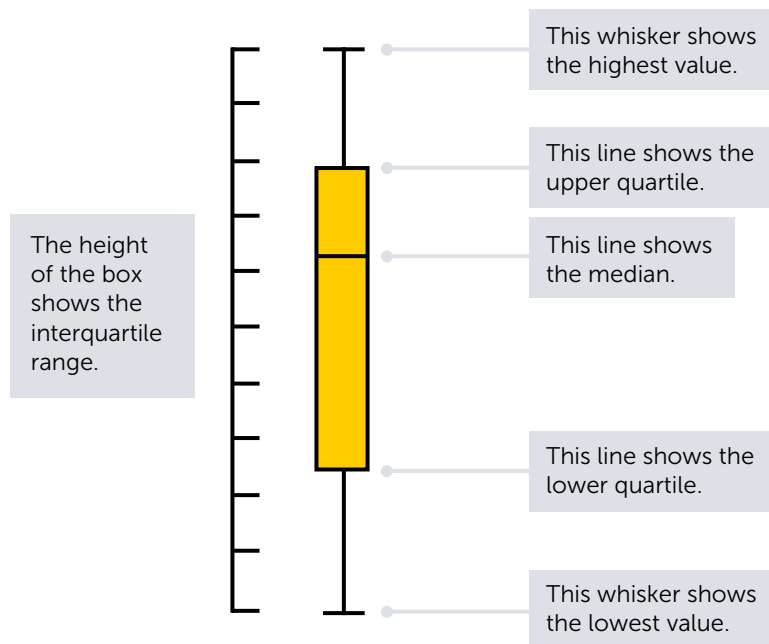
Paediatric centre

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 2a: Paediatric centre analysis

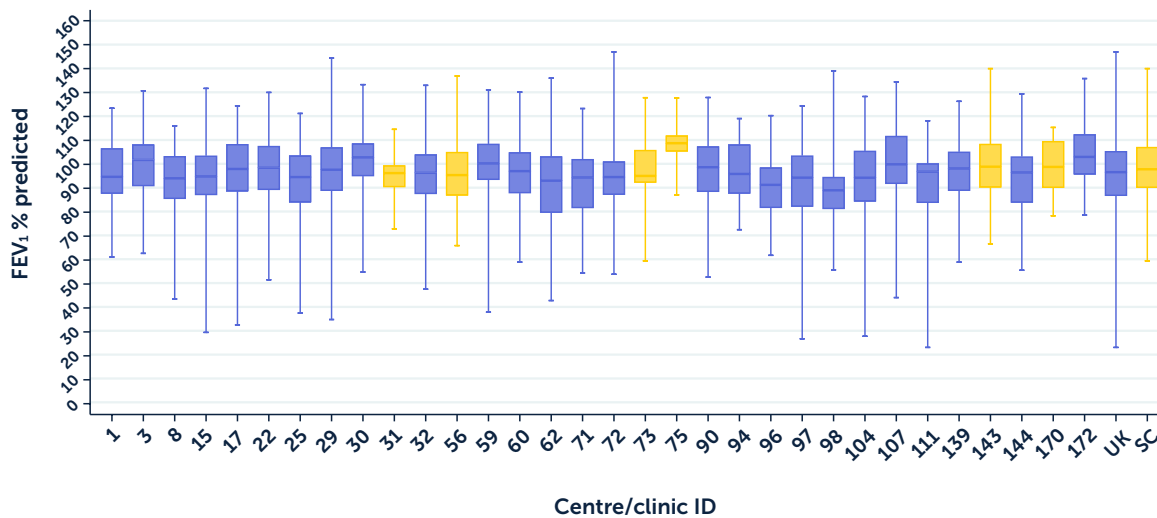


This section shows results for the five paediatric centres with their network clinics, and two stand-alone clinics.

Key

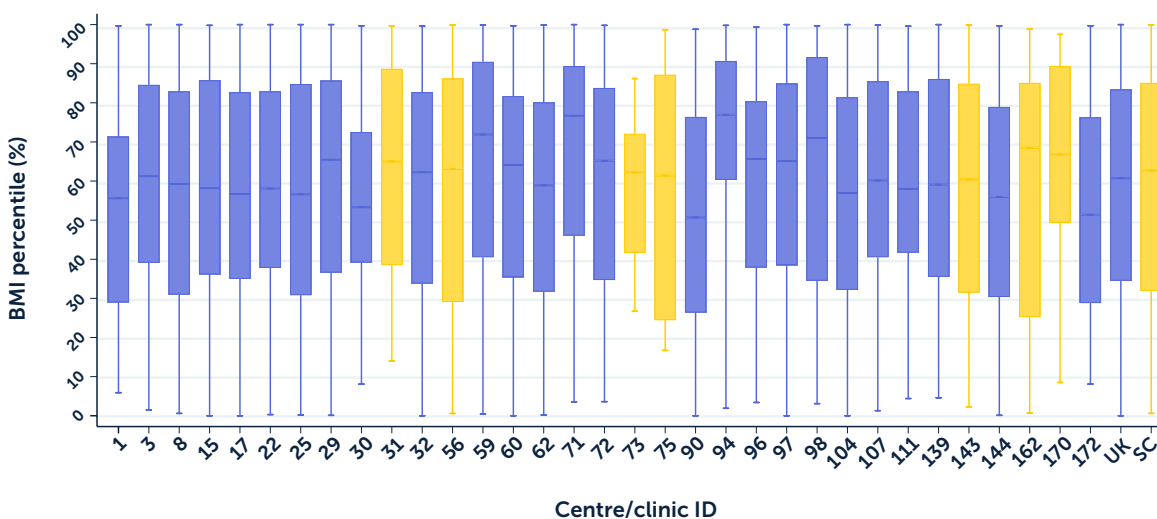
- Services in the UK
- Services in Scotland

2.1 FEV₁% predicted (GLI equations) among patients aged six and older by paediatric centre/clinic (without a history of lung transplant)



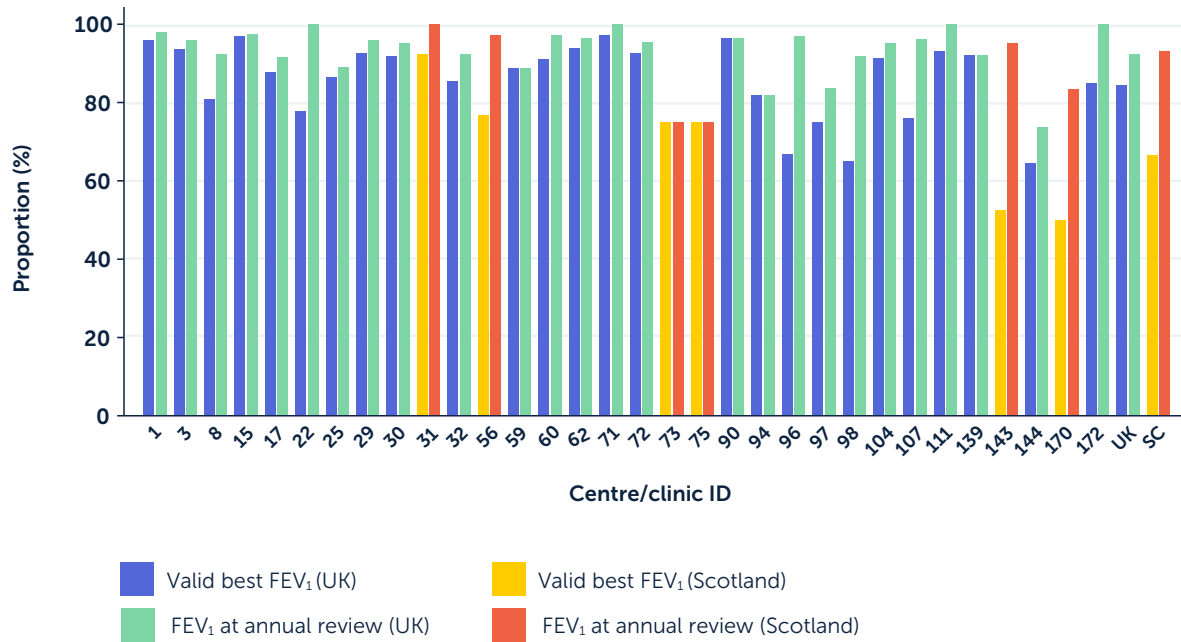
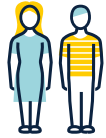
The mean FEV₁% predicted of patients attending paediatric centres/clinics in Scotland is 98.3% predicted (IQR: 90.3-107.2).

2.2 Body Mass Index (BMI) percentile among patients aged two to 15 years by paediatric centre/clinic



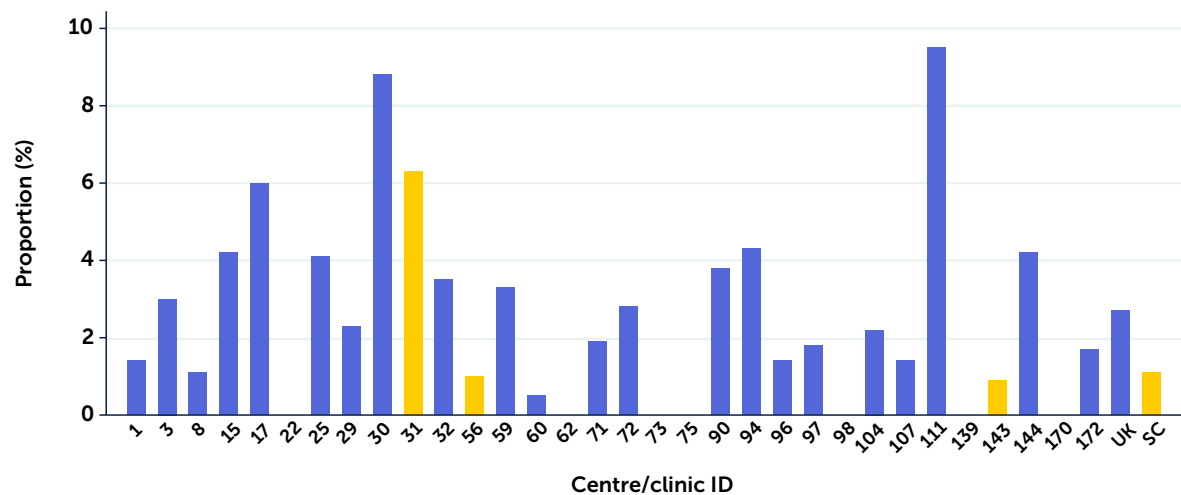
The median BMI percentile of patients attending paediatric centres/clinics in Scotland is 58.3 (IQR: 32.4-83.4).

2.3 Data completeness by paediatric centre/clinic*

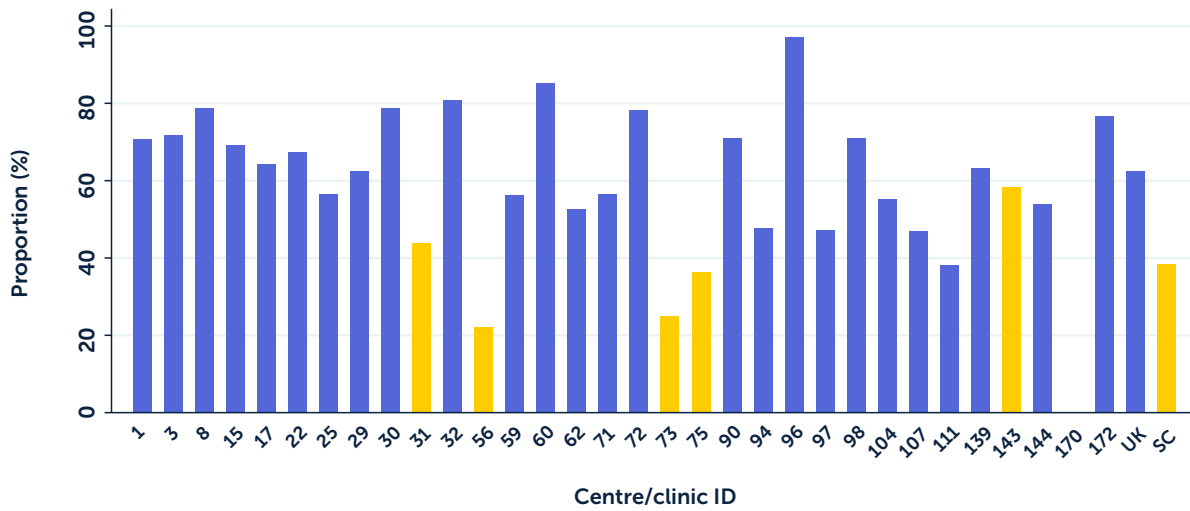
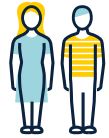


* The chart above shows the proportion of patients who had a valid best FEV₁% and an FEV₁% at annual review, excluding patients under six years of age. Best FEV₁% was considered valid if it was not missing, and the percent predicted was not more than 0.5% lower than the annual review value. For some patients there may be medical reasons why FEV₁ could not be taken, so centres may not be able to get 100% completeness.

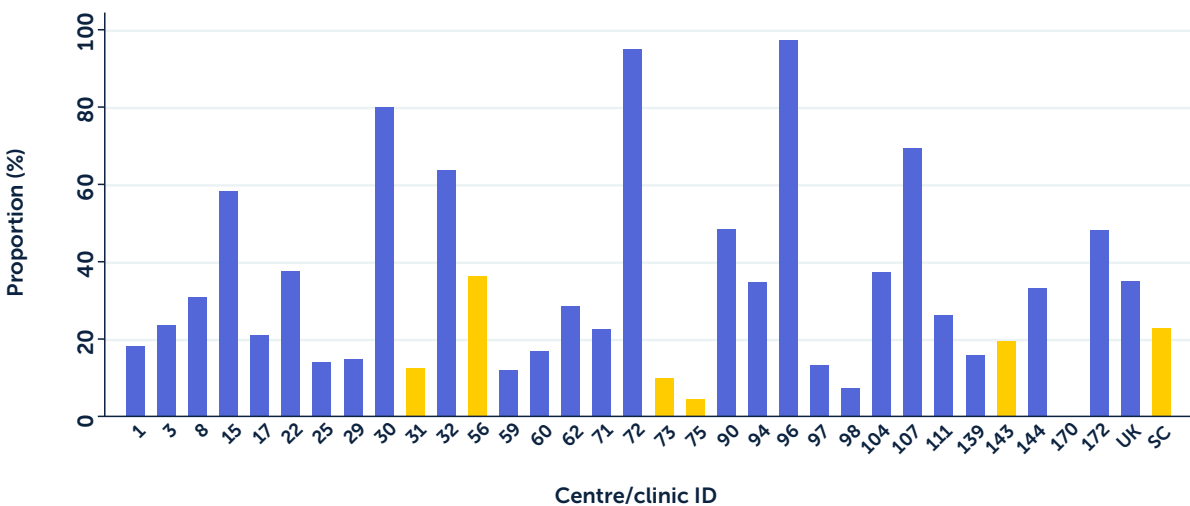
2.4 Proportion of patients with chronic *Pseudomonas aeruginosa* by paediatric centre/clinic



2.5 Proportion of patients receiving DNase treatment by paediatric centre/clinic



2.6 Proportion of patients receiving hypertonic saline treatment by paediatric centre/clinic



Section 2b: Adult centre analysis

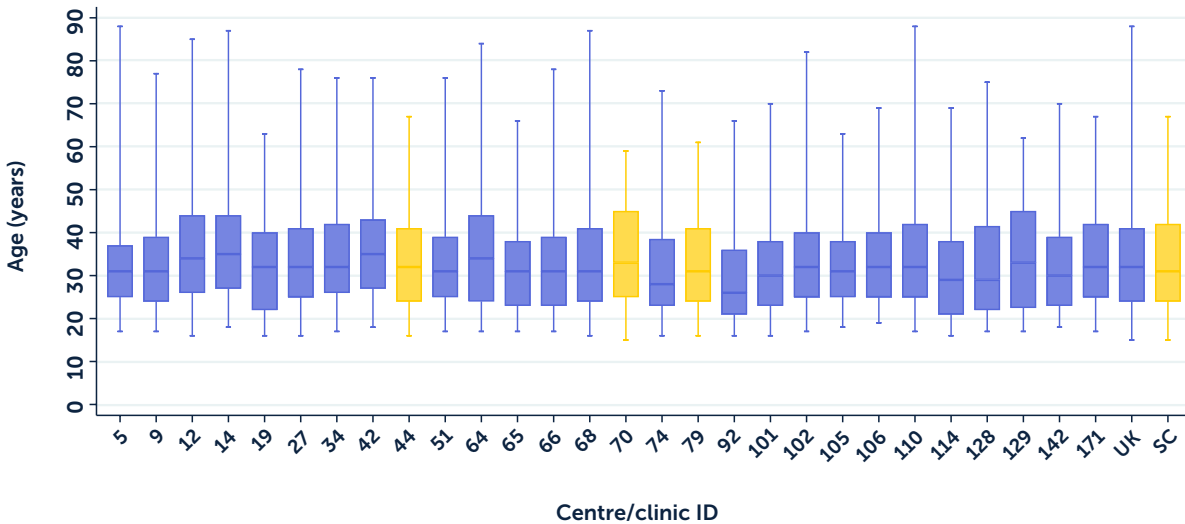


This section shows results for the three adult centres with their network clinics.

Key

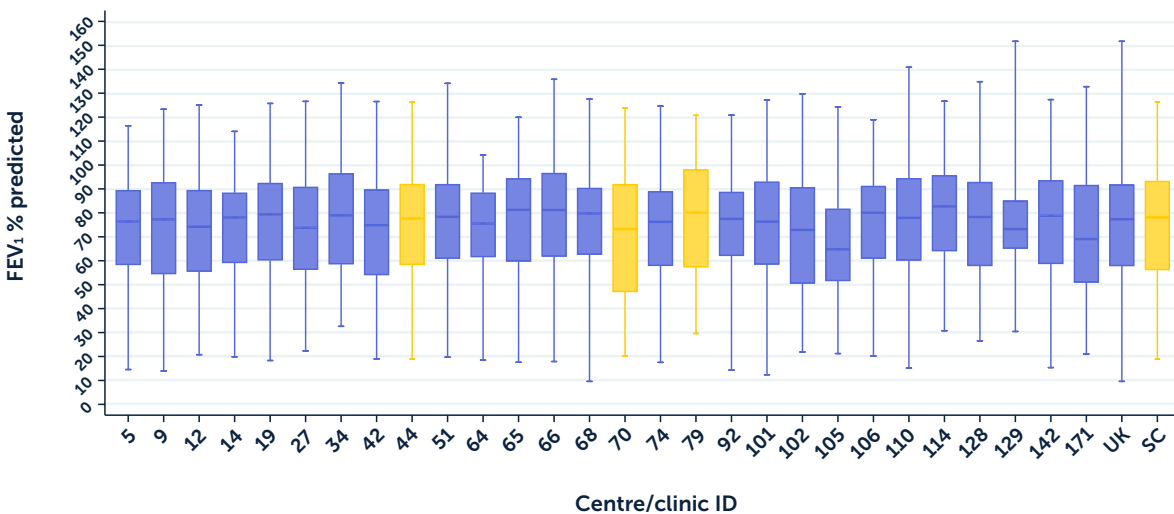
- Services in the UK
- Services in Scotland

2.7 Age distribution by adult centre/clinic



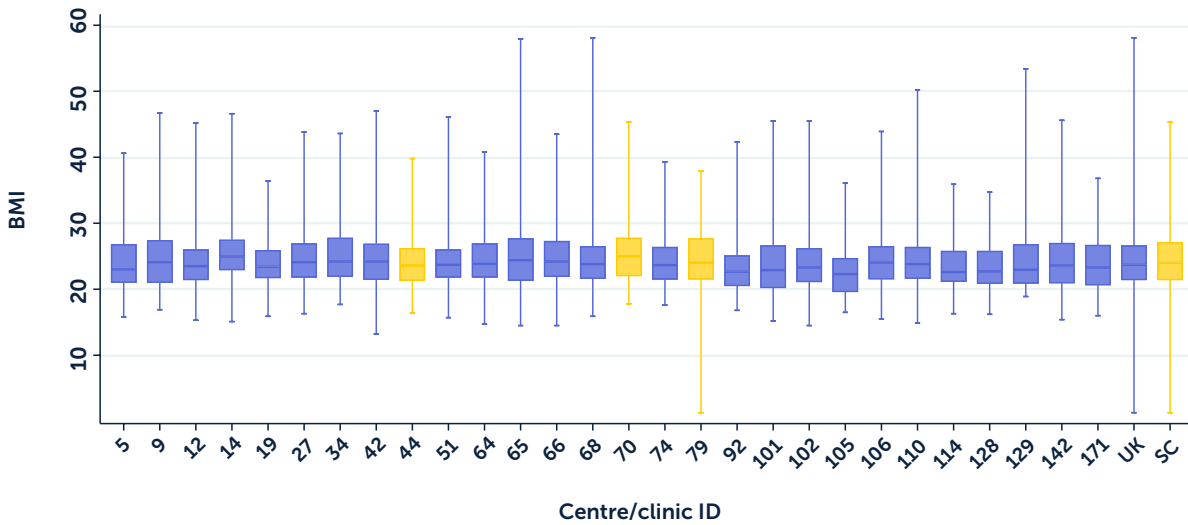
The median age of patients attending adult services in Scotland is 31 years (IQR: 24-42).

2.8 FEV₁% predicted (GLI equations) by adult centre/clinic (without a history of lung transplant)



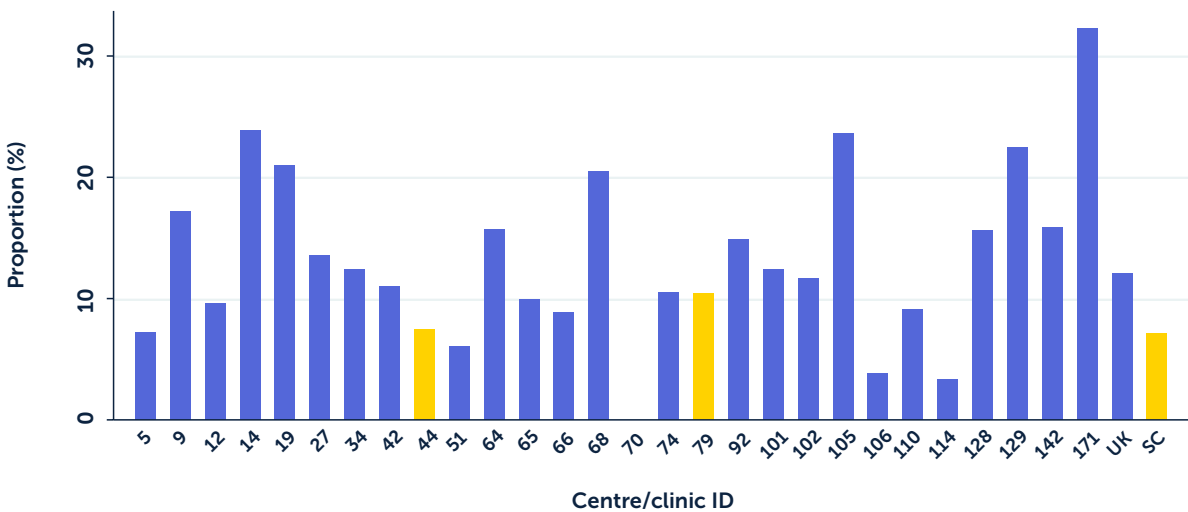
The median FEV₁ % predicted of patients attending adult services in Scotland is 78.1% (IQR 56.1-93.3).

2.9 Body Mass Index (BMI) distribution among patients aged 16 years and older by adult centre/clinic



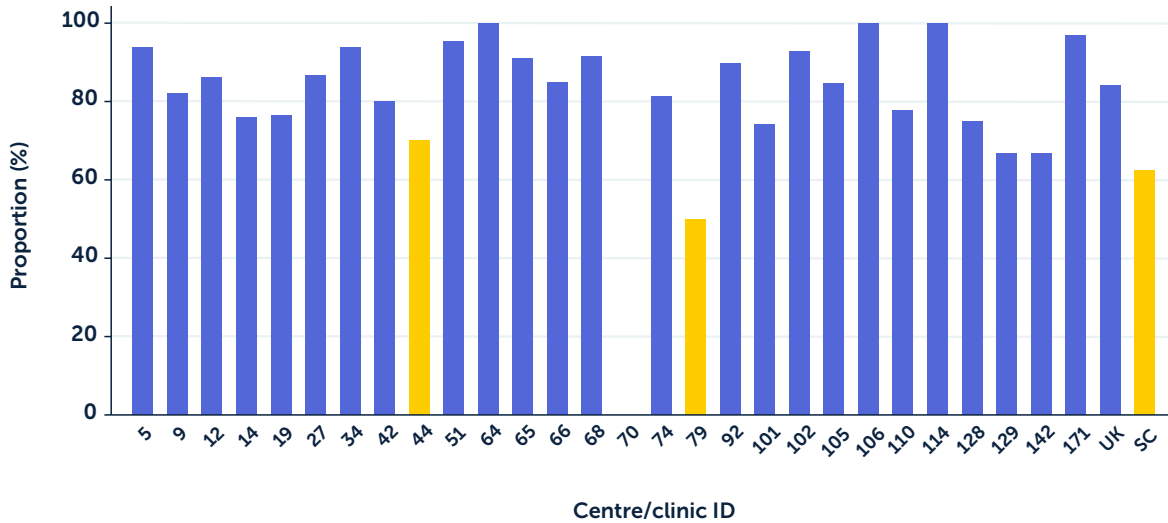
The median BMI of patients attending adult services in Scotland is 24.0 (IQR: 21.4-27.1).

2.10 Proportion of patients with chronic *Pseudomonas aeruginosa* by adult centre/clinic



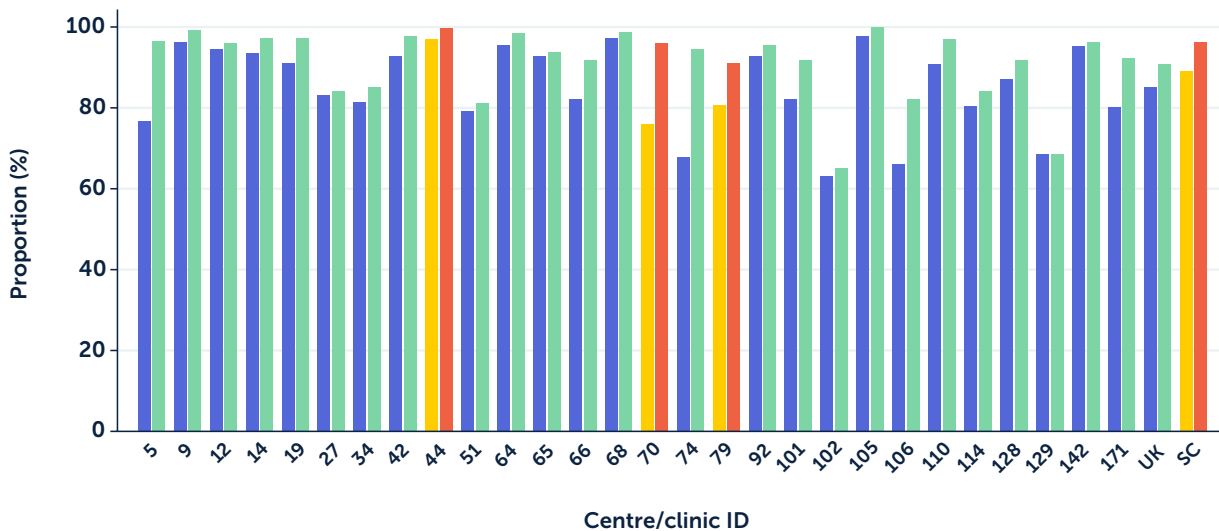
The proportion of patients attending adult services in Scotland with chronic *P. aeruginosa* is 6.3%.

2.11 Inhaled antibiotic use for patients with chronic *Pseudomonas aeruginosa* by centre/clinic



The proportion of chronic *P. aeruginosa* patients on inhaled antibiotics in Scotland is 60.8%.

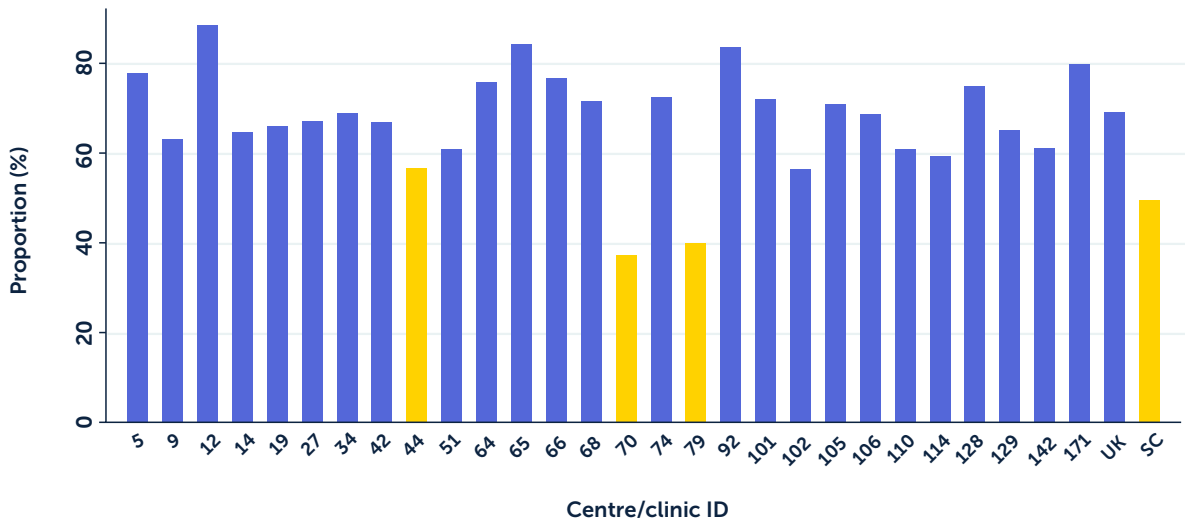
2.12 Data completeness by adult centre/clinic*



- Valid best FEV₁ (UK)
- FEV₁ at annual review (UK)
- Valid best FEV₁ (Scotland)
- FEV₁ at annual review (Scotland)

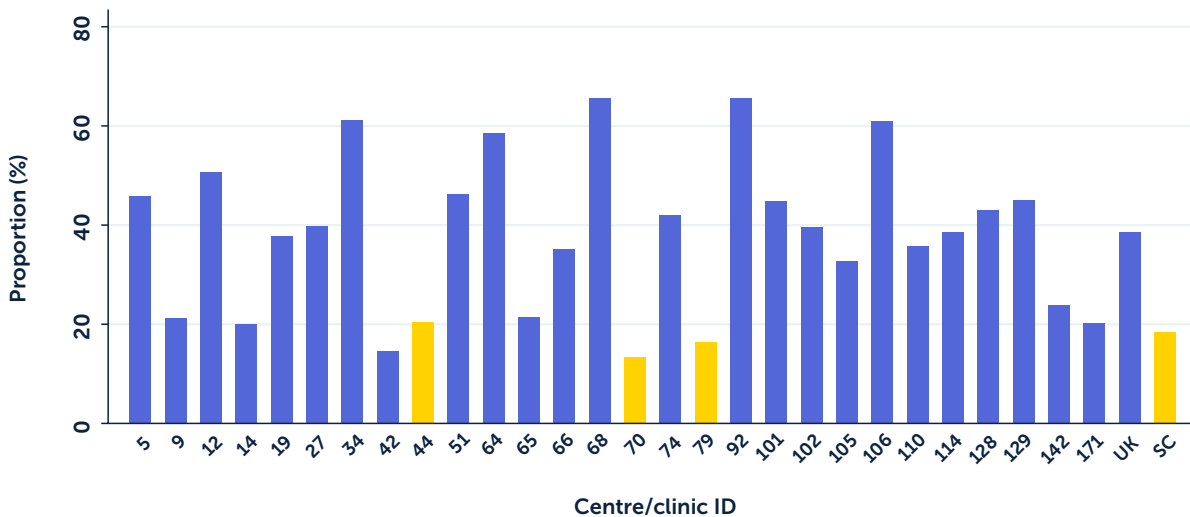
*FEV₁ was considered valid if it was not missing, and the percent predicted was not more than 0.5% lower than the annual review value. For some patients there may be medical reasons why FEV₁ could not be taken, so centres may not be able to get 100% completeness.

2.13 Proportion of patients receiving DNase treatment by adult centre/clinic



The proportion of patients attending adult services in Scotland receiving DNase treatment is 49.6%.

2.14 Proportion of patients receiving hypertonic saline or mannitol treatment by adult centre/clinic



The proportion of patients attending adult services in Scotland receiving hypertonic saline or mannitol treatment is 17.6%.

Glossary

| Word/Phrase | Meaning |
|--|--|
| 2023 | 1 January 2023 – 31 December 2023. |
| ABPA (allergic bronchopulmonary aspergillosis) | When a person develops a respiratory allergic reaction to <i>Aspergillus fumigatus</i> . |
| 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. |
| <i>Burkholderia cepacia</i> complex | <i>B. cepacia</i> 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 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 (gastroesophageal 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. |
| <i>Haemophilus influenzae</i> | <i>H. influenzae</i> 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. |
| MRSA | Methicillin-resistant <i>Staphylococcus aureus</i> 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. |
| <i>Pseudomonas aeruginosa</i> | 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. |
| <i>Staphylococcus aureus</i> | <i>S. aureus</i> 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. |

Appendix 1: Centre-level data tables



Paediatric centres/clinics providing data in 2023 – ordered alphabetically by location

| Location | Name | Clinic ID | Total Active | Number with annual review |
|-----------------|------------------------------------|-----------|--------------|---------------------------|
| Scotland | | | | |
| Aberdeen | Royal Aberdeen Children's Hospital | 75 | 29 | 22 |
| Ayr | University Hospital Crosshouse | 170 | 19 | 8 |
| Dundee | Ninewells Hospital | 73 | 21 | 20 |
| Edinburgh | Royal Hospital for Sick Children | 143 | 124 | 108 |
| Glasgow | Royal Hospital for Sick Children | 56 | 160 | 99 |
| Inverness | Raigmore Hospital | 31 | 17 | 16 |

Paediatric centres/clinics providing data in 2023 – ordered alphabetically by location

| | | | BMI | | | |
|-----------------|------------------------------------|-----------|------------|-------------------|-----------------|--------|
| Location | Name | Clinic ID | Number | Mean - unadjusted | Mean - adjusted | Median |
| Scotland | | | | | | |
| Aberdeen | Royal Aberdeen Children's Hospital | 75 | 20 | 59.8 | 59.9 | 58.3 |
| Ayr | University Hospital Crosshouse | 170 | 5 | 54.9 | 54.7 | 53.5 |
| Dundee | Ninewells Hospital | 73 | 17 | 53.0 | 53.0 | 54.6 |
| Edinburgh | Royal Hospital for Sick Children | 143 | 104 | 60.5 | 60.5 | 61.4 |
| Glasgow | Royal Hospital for Sick Children | 56 | 90 | 56.1 | 56.1 | 58.8 |
| Inverness | Raigmore Hospital | 31 | 15 | 53.8 | 53.8 | 56.8 |



| Clinic ID | Age | | FEV ₁ % predicted at annual review | | | | Best** FEV ₁ % predicted | | | |
|-----------|------|--------|---|-------------------|-----------------|--------|-------------------------------------|-------------------|-----------------|--------|
| | Mean | Median | Number | Mean - unadjusted | Mean - adjusted | Median | Number* | Mean - unadjusted | Mean - adjusted | Median |
| 75 | 7.4 | 6.5 | 9 | 109.1 | 108.1 | 108.8 | 12 | 108.2 | 107.1 | 107.5 |
| 170 | 9.9 | 12.0 | 5 | 98.6 | 99.3 | 98.9 | 5 | 101.1 | 102.0 | 98.9 |
| 73 | 9.6 | 10.8 | 12 | 97.1 | 96.6 | 95.1 | 14 | 101.1 | 100.5 | 101.4 |
| 143 | 9.8 | 10.1 | 80 | 98.7 | 98.4 | 99.0 | 83 | 101.6 | 101.2 | 101.3 |
| 56 | 8.9 | 8.8 | 67 | 97.3 | 96.8 | 95.5 | 69 | 101.0 | 100.3 | 100.4 |
| 31 | 9.7 | 9.4 | 13 | 95.5 | 95.1 | 96.3 | 13 | 100.0 | 99.4 | 99.0 |

| Clinic ID | Chronic <i>Pseudomonas</i> | | Having at least 1 IV days | | Receiving DNase treatment | | Receiving hypertonic saline or mannitol treatment | | Inhaled antibiotic use among patients with chronic <i>Pseudomonas</i> | |
|-----------|----------------------------|----------------|---------------------------|----------------|---------------------------|----------------|---|----------------|---|----------------|
| | Number | Proportion (%) | Number | Proportion (%) | Number | Proportion (%) | Number | Proportion (%) | Number | Proportion (%) |
| 75 | 0 | 0.0 | 0 | 0.0 | 8 | 36.4 | <5 | -* | 0 | 0.0 |
| 170 | 0 | 0.0 | <5 | -* | 0 | 0.0 | 0 | 0.0 | 0 | 0.0 |
| 73 | 0 | 0.0 | <5 | -* | 5 | 25.0 | <5 | -* | 0 | 0.0 |
| 143 | <5 | -* | 19 | 17.6 | 63 | 58.3 | 21 | 19.4 | <5 | -* |
| 56 | <5 | -* | 12 | 12.1 | 21 | 21.2 | 35 | 35.4 | <5 | -* |
| 31 | <5 | -* | 0 | 0.0 | 7 | 43.8 | <5 | -* | <5 | -* |

* Redacted to adhere to statistical disclosure guidelines.

** Where 'Best' values were missing, or lower than FEV₁% predicted taken at annual review, the annual review value was used.

Appendix 1: Centre-level data tables



Adult centres/clinics providing data in 2023 – ordered alphabetically by location

| Location | Name | Clinic ID | Total active | Number with annual review |
|-----------------|-------------------------------------|-----------|--------------|---------------------------|
| Scotland | | | | |
| Aberdeen | Aberdeen Royal Infirmary | 70 | 78 | 67 |
| Edinburgh | Western General Hospital | 44 | 282 | 268 |
| Glasgow | Queen Elizabeth University Hospital | 79 | 242 | 115 |

Adult centres/clinics providing data in 2023 – ordered alphabetically by location

| | | | BMI | | | |
|-----------------|-------------------------------------|-----------|--------|-------------------|-----------------|--------|
| Location | Name | Clinic ID | Number | Mean - unadjusted | Mean - adjusted | Median |
| Scotland | | | | | | |
| Aberdeen | Aberdeen Royal Infirmary | 70 | 66 | 26.1 | 26.0 | 25.0 |
| Edinburgh | Western General Hospital | 44 | 268 | 24.4 | 24.5 | 23.7 |
| Glasgow | Queen Elizabeth University Hospital | 79 | 115 | 24.6 | 24.6 | 24.0 |



| Clinic ID | Age | | FEV ₁ % predicted at annual review | | | | Best** FEV ₁ % predicted | | | |
|-----------|------|--------|---|-------------------|-----------------|--------|-------------------------------------|-------------------|-----------------|--------|
| | Mean | Median | Number | Mean - unadjusted | Mean - adjusted | Median | Number | Mean - unadjusted | Mean - adjusted | Median |
| 70 | 35.2 | 33.4 | 60 | 72.0 | 71.8 | 74.3 | 60 | 75.8 | 75.8 | 77.6 |
| 44 | 34.3 | 32.0 | 253 | 74.5 | 73.9 | 77.7 | 254 | 79.3 | 79.0 | 83.1 |
| 79 | 33.7 | 31.0 | 109 | 77.3 | 76.9 | 80.1 | 113 | 79.6 | 79.3 | 83.4 |

| Clinic ID | Chronic <i>pseudomonas</i> | | Having at least 1 IV days | | Receiving DNase treatment | | Receiving hypertonic saline or mannitol treatment | | Inhaled antibiotic use among patients with chronic <i>pseudomonas</i> | |
|-----------|----------------------------|----------------|---------------------------|----------------|---------------------------|----------------|---|----------------|---|----------------|
| | Number | Proportion (%) | Number | Proportion (%) | Number | Proportion (%) | Number | Proportion (%) | Number | Proportion (%) |
| 70 | 0 | 0.0 | 9 | 13.4 | 20 | 29.9 | <5 | -* | 0 | 0.0 |
| 44 | 20 | 10.9 | 46 | 17.2 | 136 | 50.7 | 30 | 11.2 | 14 | 70.0 |
| 79 | 12 | 11.3 | 31 | 27.0 | 36 | 31.3 | 9 | 7.8 | 6 | 50.0 |

* Redacted to adhere to statistical disclosure guidelines.

** Where 'Best' values were missing, or lower than FEV₁% predicted taken at annual review, the annual review value was used.

Appendix 2: Full list of mutations 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 | 876 | 90.1 |
| c.1652G->A | p.Gly551Asp | G551D | 95 | 9.8 |
| c.350G->A | p.Arg117His | R117H | 79 | 8.1 |
| c.1624G->T | p.Gly542X | G542X | 62 | 6.4 |
| c.200C->T | p.Pro67Leu | P67L | 54 | 5.6 |
| c.3454G->C | p.Asp1152His | D1152H | 23 | 2.4 |
| c.1679G->C | p.Arg560Thr | R560T | 18 | 1.9 |
| c.1585-1G->A | | 1717-1G->A | 18 | 1.9 |
| c.1477C->T | p.Gln493X | Q493X | 15 | 1.5 |
| c.2657+5G->A | | 2789+5G->A | 14 | 1.4 |
| c.3909C->G | p.Asn1303Lys | N1303K | 12 | 1.2 |
| c.1135G->T | p.Glu379X | E379X | 12 | 1.2 |
| c.489+1G->T | | 621+1G->T | 10 | 1.0 |
| c.3717+12191C->T | | 3849+10kbC->T | 10 | 1.0 |
| c.1364C->A | p.Ala455Glu | A455E | 9 | 0.9 |
| c.3528delC | p.Lys1177SerfsX15 | 3659delC | 9 | 0.9 |
| c.1558G->T | p.Val520Phe | V520F | 8 | 0.8 |
| c.178G->T | p.Glu60X | E60X | 8 | 0.8 |
| c.3140-26A->G | | 3272-26A->G | 7 | 0.7 |
| c.2657+2_2657+3insA | | 2789+2insA | 7 | 0.7 |
| c.1705T->G | p.Tyr569Asp | Y569D | 5 | 0.5 |
| c.3846G->A | p.Trp1282X | W1282X | 5 | 0.5 |
| c.1519_1521delATC | p.Ile507del | I507del | 5 | 0.5 |
| c.1766+1G->A | | 1898+1G->A | 5 | 0.5 |
| c.1329_1330insAGAT | p.Ile444ArgfsX3 | 1461ins4 | <5 | - |
| c.1721C->A | p.Pro574His | P574H | <5 | - |
| c.1657C->T | p.Arg553X | R553X | <5 | - |
| c.948delT | p.Phe316LeufsX12 | 1078delT | <5 | - |
| c.254G->A | p.Gly85Glu | G85E | <5 | - |
| c.579+3A->G | | 711+3A->G | <5 | - |
| c.2012delT | p.Leu671X | 2143delT | <5 | - |
| c.3468G->A | | 3600G->A | <5 | - |
| c.429delT | p.Phe143LeufsX10 | 557delT | <5 | - |
| c.1523T->G | p.Phe508Cys | F508C | <5 | - |
| c.509G->A | p.Arg170His | R170H | <5 | - |
| c.2490+1G->A | | 2622+1G->A | <5 | - |
| c.223C->T | p.Arg75X | R75X | <5 | - |
| c.1680A->C | p.Arg560Ser | R560S | <5 | - |
| c.3196C->T | p.Arg1066Cys | R1066C | <5 | - |
| c.1006_1007insG | p.Ile336SerfsX28 | 1138insG | <5 | - |
| c.2988+1G->A | | 3120+1G->A | <5 | - |
| c.1210-12[5](AJ574948.1:g.152T[5]) | | 5T | <5 | - |

| Nucleotide | Protein | Legacy name | N | % |
|--|------------------|------------------------------|----|---|
| c.1367T->C | p.Val456Ala | V456A | <5 | - |
| c.164+2T>C | | 296+2T->C | <5 | - |
| c.2052delA | p.Lys684AsnfsX38 | 2184delA | <5 | - |
| c.3737C->T | p.Thr1246Ile | T1246I | <5 | - |
| c.617T->G | p.Leu206Trp | L206W | <5 | - |
| c.3884_3885insT | p.Ser1297PhefsX5 | 4016insT | <5 | - |
| c.3276C->A or c.3276C->G | p.Tyr1092X | Y1092X(C->A) | <5 | - |
| c.292C->T | p.Gln98X | Q98X | <5 | - |
| c.933C>G | p.Phe311Leu | F311L | <5 | - |
| c.3475T->C | p.Ser1159Pro | S1159P | <5 | - |
| c.274G->A | p.Glu92Lys | E92K | <5 | - |
| c.1327G->T | p.Asp443Tyr | D443Y | <5 | - |
| c.1986_1989delAACT | p.Thr663ArgfsX8 | 2118del4 | <5 | - |
| c.54-5940_273+10250del21kb | p.Ser18ArgfsX16 | CFTRdele2,3 | <5 | - |
| c.2900T->C | p.Leu967Ser | L967S | <5 | - |
| c.2859_2890delACATTCTGTTCTTC AAGCACCTATGTCAACCC | p.Leu953PhefsX11 | 2991del32 | <5 | - |
| c.262_263delTT | p.Leu88IlefsX22 | 394delTT | <5 | - |
| c.3197G->A | p.Arg1066His | R1066H | <5 | - |
| c.1040G->C | p.Arg347Pro | R347P | <5 | - |
| c.2988G->A | | 3120G->A | <5 | - |
| c.1585-8G->A | | 1717-8G->A | <5 | - |
| c.3476C->T | p.Ser1159Phe | S1159F | <5 | - |
| c.443T->C | p.Ile148Thr | I148T | <5 | - |
| c.2051_2052delAAinsG | p.Lys684SerfsX38 | 2183AA->G or 2183delAA->G | <5 | - |
| c.3158C->T | p.Thr1053Ile | T1053I | <5 | - |
| c.1466C->A | p.Ser489X | S489X | <5 | - |
| c.3208C->T | p.Arg1070Trp | R1070W | <5 | - |
| c.349C->T | p.Arg117Cys | R117C | <5 | - |
| c.3484C->T | p.Arg1162X | R1162X | <5 | - |
| c.3705T->G | p.Ser1235Arg | S1235R | <5 | - |
| c.4147_4148insA | p.Ile1383AsnfsX3 | 4279insA | <5 | - |
| c.1538A->G | p.Asp513Gly | D513G | <5 | - |
| c.2583delT | p.Phe861LeufsX3 | 2711delT | <5 | - |
| c.(3873+1_3874-1)_(3963+1_3964-1) del | | CFTRdele21 | <5 | - |
| c.349C->G | p.Arg117Gly | R117G | <5 | - |
| c.1055G->A | p.Arg352Gln | R352Q | <5 | - |
| c.273+1G->A | | 405+1G->A | <5 | - |
| c.(53+1_54-1)_(489+1_490-1)del | | CFTRdele2-4 | <5 | - |
| c.[1210-12[5];1210-34TG[12]] | | 5T;TG12 | <5 | - |
| c.1209+1G->A | | 1341+1G->A | <5 | - |
| c.1753G->T | p.Glu585X | E585X | <5 | - |
| c.[1210-12[5];1210-34TG[13]] | | 5T;TG13 | <5 | - |
| c.233dupT | p.Trp79LeufsX32 | 365-366insT | <5 | - |
| c.1029delC | p.Cys343X | 1161delC | <5 | - |
| c.1022_1023insTC | p.Phe342HisfsX28 | 1154insTC | <5 | - |
| c.1000C->T | p.Arg334Trp | R334W | <5 | - |

Appendix 3: Legacy names lists for modulator eligibility*

List 3a: FDA list of CFTR variants potentially responsive to elexacaftor/tezacaftor/ ivacaftor¹

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

¹ https://pi.vrtx.com/files/uspi_elexacaftor_tezacaftor_ivacaftor.pdf

List 3b: potentially responsive CFTR variants according to French Compassionate Use Programme²

| | |
|------------|---------------|
| R334W | 3849+10kbC->T |
| R1066C | 3272-26A->G |
| 2789+5G->A | 3041-15T->G |
| | N1303K |

² Burgel PR et al, Eur Respir J. Feb. 2023

* Please see graphs 1.35b and 1.36 in the report which reference these lists

List 3c: CFTR variants considered suitable for tezacaftor / ivacaftor use³

| | | | |
|--|-------------|---------------|------------|
| Named variants | E56K | P67L | D110H |
| | R117C | E193K | R347H |
| | L206W | R352Q | A455E |
| | 711+3A->G | E831X | S945L |
| | K1060T | A1067T | 2789+5G->A |
| | 3272-26A->G | 3849+10kbC->T | |
| Variants with "varying clinical consequence" (VCC) | R74W | D110E | D579G |
| | S977F | F1052V | R1070W |
| | F1074L | D1152H | D1270N |

³ <https://www.england.nhs.uk/wp-content/uploads/2020/08/Urgent-policy-statement-CFTR-off-label-rarer-mutations.pdf>

List 3d: CFTR variants considered suitable for ivacaftor use³

| | | | |
|--|-------------|---------------|------------|
| Named variants | E56K | P67L | D110H |
| | R117C | E193K | R347H |
| | L206W | R352Q | A455E |
| | 711+3A->G | E831X | S945L |
| | K1060T | A1067T | 2789+5G->A |
| | 3272-26A->G | 3849+10kbC->T | |
| Variants with "varying clinical consequence" (VCC) | R74W | D110E | D579G |
| | S977F | F1052V | G1069R |
| | R1070Q | R1070W | F1074L |
| | D1152H | D1270N | |

³ <https://www.england.nhs.uk/wp-content/uploads/2020/08/Urgent-policy-statement-CFTR-off-label-rarer-mutations.pdf>

Table 3e: CFTR modulator use in people aged six years and older by genotype group⁴

| | Genotype Group | | | | |
|----------------------|-------------------|-------------------|------------------|-----------------|------------------|
| | Group 1 | Group 2 | Group 3 | Group 4 | Group 5 |
| ETI* | 261 (94.6%) | 260 (82.8%) | 22 (56.4%) | 0 | -** |
| Tezacaftor/ivacaftor | <5 | <5 | <5 | <5 | <5 |
| Lumacaftor/ivacaftor | <5 | 0 | <5 | 0 | 0 |
| Ivacaftor | 0 | -** | 8 (20.5%) | <5 | 0 |
| Never used | 13 (4.7%) | 43 (13.7%) | 9 (23.1%) | <5 | 13 (65%) |
| Total | 276 (100%) | 314 (100%) | 39 (100%) | 7 (100%) | 20 (100%) |

⁴ Fewer than five patients were excluded because their last recorded CFTRm treatment was as part of a drug trial and specific drug was unknown

* ETI is Elexacaftor/tezacaftor/ivacaftor

** Redacted to adhere to statistical guidelines

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