Cystic Fibrosis Research News

Title:
Standards for the care of people with cystic fibrosis (3rd edition; 2023-2024)

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What was your research question?

What does the best care for people with CF look like?
How do we best use the available evidence to support and empower people with CF and their families?

Why is this important?

Healthcare providers, scientists and companies look at and test ways to improve care for people with CF. They work to understand more about how CF affects the body, to develop new medicines and treatments, and to compare treatments against each other.
Sometimes the research shows clearly that a treatment or method of care improves health. Sometimes the results are less clear, and its unclear if one care option is better than others.
Medical societies like the European Cystic Fibrosis Society and patient organizations like Cystic Fibrosis Europe work to improve the care of people with cystic fibrosis. One way to do this is by working together to produce "standards of care."
This means listening to the CF community and their healthcare providers, looking at all the research results available and finding wide agreement about what people with CF need and what the best care looks like. These standards describe the level of care that people with CF may expect and that CF teams should aim for. The standards also describe what we hope the future will look like for people with CF.
These guidelines empower people with CF and their families to work with their CF teams to achieve the best standard of care.
As many people with CF can now access modulator therapy, it is a new time for the community. This makes now a good time to look at the best evidence to support care.

What did you do?

Over the past 20 years, the ECFS has published standards for different parts of CF care. The last big update was in 2018. A lot has happened in the world of CF since then. In 2022, ECFS

created a core committee to update the standards for the care of people with CF. The committee included doctors (looking after both adults and children with CF), a psychologist, a representative from the European patient organisation (CF Europe), scientists and other healthcare professionals.

The committee listed the different topics for the standards and invited experts from all over the world to write a summary about each topic. These experts, called "authors", were healthcare professionals, researchers, people working at a patient organisation and people with CF. The authors were also asked to write 1-3 statements with the most important messages for their section.

We showed the statements to hundreds of people in the CF community to ask if they agreed with the statement. This step was important to make sure that a large majority of people in the community were happy with the statement. We considered that a statement had agreement (also called consensus) if at least 80% of people agreed with the statement. Standards of care projects in other conditions also used this 80% agreement level. This makes the statements very powerful.

What did you find?

We started planning the project to update the standards for the care of people with CF in 2022. Between 2023 and the middle of 2024, 104 authors co-wrote and published 4 scientific papers, providing guidance on:

- Timely and accurate diagnosis
- Establishing and maintaining health
- Recognizing and addressing CF health issues
- Planning for a longer life

Click here to see a full index of all the topics covered in the 4 papers.

We produced 89 statements. In total 659 people from the community took a survey to review the statements. All the statements had strong agreement (mostly well over the 80% agreement threshold).

Click here to read the 89 consensus statements from the papers.

The first paper is called **Timely and accurate diagnosis**. It reminds readers that quick and accurate diagnosis is still highly important. Genetic analysis is becoming more and more useful and is available in more hospitals. This genetic analysis is being used alongside newborn bloodspot screening, or NBS for short. High quality NBS is essential for early diagnosis. For the first time, we recommend that expert diagnostic hubs are set up in each country/region, to make sure that diagnosis is fast and accurate. CFTR modulator medicines are only approved for people with CF with certain variants in their *CFTR* gene. This means that it is even more

important to accurately diagnose CF and the specific gene variants that cause it. The paper also explores how to handle unclear diagnoses.

The second paper is called **Establishing and maintaining health**. It covers well established aspects of CF care, including excellent nutrition, airway clearance, and exercise. It describes how health can be maximised, instead of looking at health as a series of problems to treat. This is a new way to look at the CF life journey. The paper also reminds us how important "partnership working" is. People with CF and their CF teams should work together to decide on health goals. This helps people with CF to eat well, breathe freely, avoid cigarettes and e-cigarettes and have an active healthy lifestyle. Remote care and monitoring might reduce the number of hospital visits for some people, but face to face clinic visits remain important. CFTR modulator medicines should be available to all eligible people with CF.

The third paper is called **Identifying and addressing CF health issues**. It covers the unique health problems faced by people with CF, like CF related diabetes and liver disease. It is still important to monitor, identify and treat airway infection and inflammation, even for people on CFTR modulator therapy. CF teams support people with CF in their emotional and psychological journeys. Life can become challenging when more and more treatment is needed, sometimes including transplantation. People with more advanced CF disease need holistic care adapted to what each person needs. This should balance quality of life, treatment burden and health results. Finally, we discuss how CF teams support and care for people with CF through advanced disease and end of life.

The fourth paper is called **Planning for a longer life.** The outlook is changing for people with CF. New treatments and improved health could lead to better life expectancy. This is why the standards now include information about planning for a longer life. This paper supports people with CF to balance their chronic condition with leading a full and active life. We discuss issues like school and further education, working, living independently, dealing with "life admin" (insurance, pensions etc) and retirement. We discuss how CF impacts planning for and raising a family. We consider how global inequity and poverty can impact the care of people with CF. We look at the relationship between CF and our changing planet. We also discuss the values and beliefs of people with CF and how this relates to their care. As some people with CF reach older age, they will experience the non-CF health issues of aging. Older people with CF must also participate in health screening that is available to the whole population. Some screening (such as for bowel cancer) is particularly important for people with CF. Finally, we discuss research and registries, which are very important topics for the CF community. People with CF should have opportunity to shape and contribute to research. Partnership working between the CF team and the person with CF is key to achieve their hopes and aspirations.

What does this mean and reasons for caution?

The arrival of CFTR modulator treatment changed CF for many people. It also changed how CF teams care for people with CF. Various therapies and medical procedures that have been standard practice for many years are now being re-evaluated due to the recent advancements. This means that new studies are needed, but this takes time. Clear communication with the CF team has always been important for people with CF. This is even more important while researchers gather more evidence about modulator therapy, along with partnership-working and shared decision making. People with CF and their CF teams must discuss and share decisions on issues like reducing some supporting therapies or continuing CFTR modulator therapy during pregnancy.

Some people with CF are not eligible for modulator therapy. This is because modulators do not work for all gene variants that cause CF. However the guidance in these papers is relevant for all people with CF, whether they are on modulator therapy or not.

Not all people with CF receive the level of "standard care" described in these papers. There are many reasons for this. Healthcare systems in different countries have different budgets. The budget can be tight, especially in lower- and middle-income countries. Sometimes the budget does not pay for expensive medicines or procedures. This means that some people who are "eligible" for a medication may not have access to it. It often takes time before healthcare budgets "catch up" with research discoveries and agree to fund new treatments. But all CF teams should take steps to improve care and aim to deliver the standards of care we describe.

What's next?

Standards help healthcare teams deliver high quality care to people with CF. These papers also empower people with CF and their families to work with their healthcare teams to define their goals and hopes. The ECFS will work with CF teams all over Europe to support them to put these standards of care in place. It can be challenging for some CF centres to establish high quality care. These standards will help them to access the resources and funds they need, with the help of the ECFS and patient organisations.

For people with CF or parents, the standards of care can help understand what high-quality care looks like. The standards might also be a starting point for a conversation with the medical team on the care received. But before people can really start using these standards of care, they have to know they exist, and be able to understand them. This summary is helping to increase awareness of these new standards.

Importantly, healthcare professionals and patient organisations can use the standards of care to ask their governments for more resources for CF. For example, governments could look at

the standards of care and decide to pay for more healthcare providers (doctors, nurses, dieticians etc.) and new medicines (like CFTR modulators).

Where can I read the scientific articles?

The articles are free to read at the following links. You can also look at our index here, explaining what topics are discussed in each paper.

- Timely and accurate diagnosis
- Establishing and maintaining health
- Recognizing and addressing CF health issues
- Planning for a longer life