

Kaftrio – complex and individual experiences

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Introduction

This factsheet contains information for people with cystic fibrosis and their families, whether they are taking Kaftrio or not.

Many people with cystic fibrosis (CF) aged 12 and over in the UK have been taking the CFTR modulator therapy Kaftrio since the licensing approval in June 2020, or before if they've been on a Kaftrio clinical trial. This was extended to eligible children and young people in early 2022.

We now have a better understanding of the impacts that Kaftrio can have on people in the CF community. However, it is still a relatively new therapy and more information will become available as our knowledge improves, particularly for younger age groups.

For many of those who have been taking Kaftrio, the effect on their physical, social and emotional wellbeing has been wholly positive. For others, taking Kaftrio has been more mixed, and they are surprised to experience neutral or negative thoughts, feelings and changes as well as positive ones. This is understandable – change can come with loss as well as gain.

I am not taking Kaftrio

There are many reasons why someone with CF may not be taking Kaftrio. Some of these are explored below.

You might be feeling some resentment on hearing about the benefits of Kaftrio from others with CF. Some have said they experience guilt for having these feelings, just as those who are taking Kaftrio may feel guilty that some in their community are currently feeling left behind.

You might find it useful to share your feelings with someone. Your CF team will know that you aren't taking Kaftrio and will want to support you in any way they can.

I cannot benefit from Kaftrio due to the CF gene variants I have

Someone's genotype – the CF gene variants (mutations) they have – is one reason that they might not be able to benefit from Kaftrio.

Kaftrio is only approved for people with CF who have specific CF gene variants, because it does not work for other variants. You can read more about Kaftrio and gene variants on the **Cystic Fibrosis Trust website**.

Those who are still waiting for treatment for their gene variants may be experiencing a mixture of emotions, including frustration, sadness, anger and worry. It may also be difficult to explain to well-meaning friends and family who have heard about Kaftrio in the news or on social media, that you or someone close to you are not in fact able to benefit from it.

There are also some rare variants that we do not know much about, including whether a person with these variants could benefit from Kaftrio. Over time more people with CF may be able to benefit from Kaftrio as clinical trials continue to show health improvements for additional groups.

As many people are aware, major treatment breakthroughs are still needed for some people with CF. Research into new treatments for those who cannot benefit from Kaftrio is already happening. You can find more information about this **here**.

My child is too young to take Kaftrio

In the UK, people with CF under the age of 6 with CF gene variants that would benefit from Kaftrio are currently unable to take it, unless they are involved in a trial or under compassionate use. This may change in the future as more trials are being done in younger age groups taking Kaftrio.

If you have any questions or concerns, your child's CF team will do their best to answer them.

There are medical reasons which mean I can't benefit from Kaftrio

There will be some people who have the right genotype who might not be able to take Kaftrio for other reasons. Some people are taking medications that might interact poorly with Kaftrio, such as immunosuppressants after an organ transplant. Currently Kaftrio, and other modulator therapies, are not recommended for most people who have received a lung transplant. This is because most of the benefits from the treatments are seen in CF lungs and are not seen in new transplanted lungs. Some people who have had organ transplants and then started on Kaftrio have experienced too many side effects to continue taking it.

More research is needed to better understand the benefits, risks, and the impact Kaftrio could have for people who have had organ transplants. If you have had an organ transplant, you, your CF team and transplant team can decide together whether the risks of taking Kaftrio would outweigh the benefits. You can read more on the **Cystic Fibrosis Trust website**.

If you have had a lung transplant there may be other complex feelings about Kaftrio becoming available at this point in your life, and not a few years earlier. Your CF team will be able to offer psychological help if this is affecting you.

I've had to stop taking Kaftrio

Since Kaftrio was made available, a small number of people with CF have been medically advised not to take it or have made the decision to stop taking it due to physical health challenges, such as severe liver disease.

A small number of people with CF have also reported that their mental health has changed since starting Kaftrio, or have experienced other difficult symptoms such as 'brain fog' or forgetfulness. Some have changed the way they take Kaftrio, or stopped taking it altogether.

If you think you might be experiencing any negative changes that began after starting Kaftrio, make sure you speak to your CF team straight away. If the changes are to your mental health, see also the section, '**Mental health and other negative changes**'.

I am unsure if I can, or should, take Kaftrio

For a small number of people with CF, there may still be some uncertainty about whether you will be able to benefit from Kaftrio, perhaps because of your CF gene variants. Waiting for a decision from your CF team or others may be understandably stressful and frustrating. Some people with CF have described a feeling of 'losing ground' if their health is worsening while they are waiting to hear if they can take Kaftrio. Your CF team will be working hard to make sure that they find out as soon as possible. Speak to them if you have any questions or worries about this.

On the other hand, even if you know that you can benefit from Kaftrio, hearing about other people's experiences may mean you are unsure about taking it yourself, or whether your child should start taking it.

This decision is complex and individual. The benefits of taking Kaftrio outweigh any potential drawbacks in most cases, but please reach out to your CF team with any concerns you have – they will be able to help you choose an option that is right for you.

I am taking Kaftrio

The rest of this factsheet highlights many of the complex and individual experiences of people with CF in the UK who have been taking Kaftrio. As time goes on, we are learning more about how it impacts people's lives.

Remember, you might experience some, all or none of the feelings on the following pages – there is no right or wrong way to feel.

Dealing with mixed emotions

You and your family may have waited for something that treats the underlying cause of CF, rather than just the symptoms, for a long time. When you or someone close to you started taking Kaftrio, you may have felt relief, joy, gratitude, impatience for the expected benefits and hope for the future. Alongside these welcome feelings, some with CF have also experienced other feelings. These may be different for everyone, but some of them may be unexpected and uncomfortable.

If feelings like this last a long time, or are interfering with your life or with doing your treatments, do speak with your CF team. They can support you.

Some unexpected things you may have experienced include:

- **A changed relationship with CF** – After starting Kaftrio, how you see yourself and your CF may have changed. Some people have described realising that CF may not have such a big place in their life in the future. While you might have wished for this, some have said that letting go of what you have been used to can also be surprisingly hard. Most people with CF have grown up with it as part of their identity. Questions about the future or feelings of 'who am I now?' can be unsettling and you might need to give yourself time to adjust.
- **'Survivor guilt'** – This can be a common emotion when someone feels they have an opportunity that others do not have, even if they don't know them personally. This can be difficult to explain to others.
- **Wondering about the future** – If your health has improved a lot, and you feel that CF is going to have less of an impact on your life, you may have new hopes about the future. This can be exciting, but can also feel quite scary or unsettling, especially if your confidence has been knocked by health issues or other life stresses. You may need time to adjust to a changed view of the future. Those around you may not realise that the possibility of new opportunities can feel very complicated.
- **Thinking about the past** – Having more hope for the future may have changed how you think about your past. You may think about decisions you have made and how things might have been different if you had been able to take Kaftrio earlier. You may find you have more time to think about periods of illness in the past, and only now process how hard or traumatic your experience of CF was. Time to process these emotions is very important.

- **Managing disappointment** – Kaftrio affects everyone differently, and if you or someone close to you has not experienced the benefits you hoped for or expected, it's natural to feel disappointment, worry, anger or other emotions. You may even feel a sense of grief or loss at not benefiting from Kaftrio in the way you had imagined. There is ongoing research into why some people benefit more from Kaftrio than others, but it might take some time to get answers. It is important to remember that feeling these emotions does not make you ungrateful, it is understandable that you would feel this way.
- **Long-term uncertainty** – Some people have mentioned experiencing relief at being able to benefit from Kaftrio, followed by worry about how long the benefits of the treatment will last. As Kaftrio is still a fairly new treatment, we do not yet have studies that tell us the long-term benefits of Kaftrio, and it is likely to depend on your individual health and experience. It can be frustrating, but your CF team may not be able to answer this question just yet.

Managing health changes from Kaftrio

- **Adjusting to a new normal** – Kaftrio may have changed your CF symptoms in ways that are very welcome, but like many things, this can be complex. If your CF symptoms have improved, it might take time to understand the changes in your physical health, and know how to respond to them and live with them. You may be feeling less in control and struggling to recognise signs of exacerbations, or generally that you have lost some confidence in managing your CF. Your CF teams will also be adjusting, and you will need to work together to monitor your health and decide if any changes to your care are needed. See the section below, '**Changes to your CF care**' for more information.
- **Recognising changes** – For some the changes from Kaftrio can be instant, whereas for others improvement in health is gradual, or their health simply stabilises. You may find it difficult to recognise improvements or stability until you look back. It could be helpful to look back through your clinic records or even keep a diary.
- **Becoming ill while on Kaftrio** – Although most people's health while taking Kaftrio has been much better, it is likely some will still have periods of being unwell. This can come as a shock after feeling better and you may feel angry or disappointed if this has happened to you. While Kaftrio has greatly reduced the number of exacerbations that people who are taking it experience, it has not stopped them completely.

- **Weight gain** – Many people with CF have gained weight since starting Kaftrio. Some have welcomed this change, but others have not felt comfortable with it. If you have been used to your weight and eating behaviours for a long time and this changes, it can feel very difficult, even if it brings you to a healthier weight. Some people have had to adjust their diet and lifestyle to stop them from gaining too much weight. Making such changes after years of managing CF in a certain way can be hard and may take lots of trial and error. Be patient and talk openly to your CF team if you are struggling with weight gain or eating habits. If weight gain from Kaftrio is causing you major concern, you might benefit from more specialised help – your CF psychologist can refer to other services, and you may also benefit from resources from **BEAT**. You can also read the Cystic Fibrosis Trust’s resource on **‘Body image and cystic fibrosis’**.
- **Mental health and other negative changes** – A small number of people who have been taking Kaftrio have reported experiencing negative changes. These have included physical changes like headaches, as well as others such as brain fog, forgetfulness, and negative changes in mental health. For many, these changes do not last long, but others continue to experience them whilst they take Kaftrio. Talk to your CF team if you are struggling with any negative changes, even if you’re unsure whether Kaftrio is causing them or not. Together you can make a plan that is right for you. It can often be hard to know if possible side effects are related to Kaftrio or to something else, or a combination. We are awaiting more data about this from clinical trials. It’s important that these possible side effects are monitored so improvements can be made for you and the whole CF community. You can also report any potential side effects at the **MHRA Yellow Card website**.

Dealing with reactions of family and friends

Family and friends who don’t have CF may find it hard to understand the more complex emotional impact of taking Kaftrio. Sharing this factsheet with them might help them to understand that it can sometimes be a little more complicated than it seems.

- **Changing relationships** – If your health has improved a lot, other people’s expectations of you may also change. People may start to see you differently, and this can cause your relationships to change. You may find that your view of the future has changed, and that your view of the relationships in your life change with it. These changes might be very welcome, or they might create extra pressures. Like anything else, some time may be needed for you and others close to you to adjust.
- **Remaining challenges with health** – Although Kaftrio may have brought many CF health benefits, there are still remaining challenges. People close to you might think that Kaftrio is a cure and might not understand that you still face challenges with your CF, or still have to do most, if not all, of your daily treatments. On the other hand, it can be hard if you feel great but those close to you are unable to see invisible improvements in your health. It might be helpful to be honest with them about how their assumptions make you feel and what the reality of taking Kaftrio is for you.

- **Remaining challenges in your life** – Anyone can experience life problems at any time. Feeling physically better while taking Kaftrio does not mean that life will be easy. Normal life challenges, such as going through adolescence, difficult life events, stresses and worries will still happen, and you may even have more time to focus on these if you are not focused as much on your physical health. You can seek support at any time if you are struggling, from your CF team, CF psychologists or your GP. See also the details of the **Cystic Fibrosis Trust Helpline** at the end of this factsheet.

Changes to your CF care

- **Changing treatments** – Lots of people taking Kaftrio have noticed improvements in many areas of their health. With these improvements, the way their CF is treated has changed in some cases. Some people report that the 'burden' of other CF treatments can seem greater now that they are taking Kaftrio. Some even feel that some of their treatments are not needed at all now. If this is the case for you, please speak to your CF team instead of stopping your treatments. Remember, your CF team are also learning about the best way to support people who are taking Kaftrio and will try to make sure your treatments are as manageable as possible. There is also ongoing research into how Kaftrio might affect which treatments people with CF need to take, including on the **CF STORM trial**. This research is likely to take some time, so your team won't have answers immediately.
- **New procedures** – For the first year after starting to take Kaftrio, extra blood tests will be needed to monitor liver function. For some this is not a problem, but for others this can be hard. If this is an issue for you or your child, please discuss this with your CF team. Being monitored for side effects that you won't notice yourself may also be stressful, but your CF team will let you know if they have any concerns at all about results from their monitoring. Please do tell your CF team if you are struggling with the increased monitoring of your health.
- **Fewer hospital stays** – Many people have needed fewer hospital stays since starting Kaftrio. For some people this isn't a huge change, while many will be glad to spend less time in hospital. Although hospital stays come with their own stresses, they can also help you to rest and give you a chance to recharge. For some, fewer hospital stays will be challenging. CF clinic appointments may also become less frequent over time, and while this can be welcome, some may be surprised to miss the contact with their CF teams, or experience anxiety that their health is being less closely monitored.
- **Adherence** – How well people take all of their treatments (called 'adherence') varies hugely from treatment to treatment, person to person and at different times over a person's lifetime. Compared to other CF treatments, Kaftrio is relatively quick and easy to take, but some people may still struggle to take every dose, or take it with a fat-containing meal. Feeling better while taking Kaftrio has made it easier to keep up with treatments for many, but some say that certain treatments now seem like an unnecessary burden. Talk with your CF team if you are struggling to take Kaftrio or any of your treatments – they will work with you, not judge you.

Parents and family of people with CF

For parents of young people with CF who are taking Kaftrio, your role in your child's life, ideas of the future and your identity as a 'CF parent' may also change as your child's CF care changes. It might be very difficult to have to make this adjustment. Your child's CF team can help you process these changes.

Other family members, as well as partners and children of those with CF, might experience these feelings too. While improvements in the health of a family member will be very welcome and something we've all wished for, it is worth remembering that any big life change can take time to navigate and adapt to.

Impacts on education, work and finances

Having a lifelong progressive condition like CF, you may have assumed that life would go a certain way. You may have assumed that you would not need to think about working, benefits, pensions, education, housing, living independently or starting a family before starting Kaftrio. Now realising that you might need to think about practical concerns of these things, maybe for the first time, can be exciting but understandably stressful. You may be unsure what your rights are when applying for or renewing benefits, or when asking for your employers to make reasonable adjustments for you at work.

Getting your head around these changes is a huge task and can feel overwhelming. It might help to think about what you can achieve in the short and long term. This will help you to create a plan and feel prepared and reassured moving forward.

If you are worried about any of these issues, you can ask for support from your CF social worker, or by contacting the Cystic Fibrosis Trust Helpline. They will support you to make your own individual decisions, in your own time. You can find out how to contact the Helpline at the end of this factsheet.

This support might include:

• Help with benefits and financial support

- Telling you about the different benefits available if there have been changes in your physical or mental health.
- Helping you make a financial plan for any changes in your health, work or benefits, considering your lifestyle and family.
- Supporting you in talking as a family / household when making decisions.
- Exploring short- and long-term financial support.

• Education and employment

- Supporting you with applying for further education, training or a change of employment if your physical health has changed.
- Giving you information about local and national support services, and helping you to decide what works best for you and your family.
- Helping you to understand your rights and obligations (and those of your employers, school or university) and helping you to exercise these rights if needed.

- **Getting work-ready**

- Making sure you are aware of local and national support available to you in searching for work and getting work-ready if you have not worked in a long time or at all.

- **Family planning**

- Becoming a parent is always a big decision. Since Kaftrio has become available many more people with CF have become parents.
- Your CF team and the Cystic Fibrosis Trust can help you to make the best decisions for you and your family.

- **Setting up a pension**

- Supporting you with setting up a pension, depending on your past and future work.
- Telling you about different pension schemes you may be able to use.

You don't have to make any decisions alone or immediately if you don't feel ready, especially with the emotional challenges and changes you may be going through.

Accessing support

Difficult emotions usually pass after a while and are a part of healthy adjustment to change or stress. Sometimes support from family and friends, good self-care and time are what is needed to help you cope with stressful events or life changes.

If you or your child are having a difficult emotional reaction to taking Kaftrio, or you are not taking Kaftrio and this is affecting your emotional wellbeing, please reach out to your CF team. You will not be the only one they will have heard from, and the specialist CF clinical psychologists and CF social workers are ready to help at any time. They can also help those close to you understand how any changes have impacted you all.

The Cystic Fibrosis Trust is also here to support you, and you can find contact details for the Trust's **Helpline** at the end of this factsheet if you would like to speak to someone.

You can also find some good general advice and tips about emotional health and looking after yourself well on the NHS website:

- **Information on mental health and wellbeing**
- **Tips to improve your mental health and wellbeing**

Further information

Find more information resources about living with cystic fibrosis at cysticfibrosis.org.uk/information.

Our Helpline is open 10am – 4pm Monday to Friday. It's available to anyone looking for information or support with any part of cystic fibrosis, a listening ear, or just to talk things through.

You can contact our friendly team by:

- phoning **0300 373 1000** or **020 3795 2184**

If you are worried about the cost of the call please let us know and we'll call you back.

- emailing helpline@cysticfibrosis.org.uk
- reaching out on all our social media channels

Visit cysticfibrosis.org.uk/helpline for more information.

We welcome your feedback on our resources.

You can also ask for this resource in large print or as a text file. Email infoteam@cysticfibrosis.org.uk.

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This information was originally written in November 2020 by some of the members of the UK Psychosocial Professionals in CF group (UKPPCF) which includes specialist CF clinical psychologists and social workers from CF centres across the UK. People with CF and their families, medical consultants and colleagues at the Cystic Fibrosis Trust have also contributed. This information is regularly updated by the Cystic Fibrosis Trust. Last updated in March 2022.

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The information in this resource does not replace any advice from your doctor or CF team. It is important that you seek your team's advice whenever you want to change your treatment.

Cystic Fibrosis Trust

Cystic Fibrosis Trust is the charity uniting people to stop cystic fibrosis. Our community will improve care, speak out, support each other and fund vital research as we race towards effective treatments for all. We won't stop until everyone can live without the limits of cystic fibrosis.

Since 1964, we've supported people with cystic fibrosis to live longer, healthier lives – and we won't stop until everyone can live without limits imposed by CF.

cysticfibrosis.org.uk

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