



Trikafta™ Approval: Community Frequently Asked Questions

Last updated: October 25, 2019

About Trikafta™

Q1. What is Trikafta?

- Trikafta is a “triple-combination therapy” consisting of three different modulators -- tezacaftor/ivacaftor (which make up Symdeko®) combined with elexacaftor. Modulators work by helping to fix defective CFTR protein. Trikafta has the potential to be significantly more effective than current modulators.

Q2. Who is eligible to take Trikafta?

- Trikafta has been approved for people with CF ages 12 years and older who have at least one copy of the F508del mutation, regardless of their second mutation.

Q3. How soon will Trikafta be available to people with CF?

- We understand and share your sense of urgency to be able to start taking Trikafta as soon as possible. After a new drug is approved by the FDA, there are many factors that affect how much time it takes to have the drug in-hand, including when you and your doctor decide to start the therapy and your insurer's coverage policies and processes.
- Insurers will often conduct a formal review process to add new drugs to their lists. As a result, a few insurers may cover the

drug within a few weeks, but in most cases it will take many public and private insurers 3 to 6 months to review the drug and add it to their covered drug lists.

Q4. What are the potential benefits of taking Trikafta?

- Clinical trials of Trikafta showed dramatic improvements in key measures of the disease.
- People with two copies of the F508del mutation had a 10 percent increase in lung function compared to treatment with the modulator tezacaftor/ivacaftor (Symdeko®), and people with one copy of F508del had more than a 14 percent increase in lung function compared to placebo.
- Participants also saw a significant increase in the assessment used to measure individuals' quality of life as well as sweat chloride.

Q5. What effect does Trikafta have on other parts of the body?

- Clinical trial data show that many people taking highly effective modulator therapy will have improvements in lung function and gastrointestinal (GI) issues. However, advanced damage that has already occurred in the lungs and other organs (such as the pancreas) generally cannot be reversed.
- A new clinical study called PROMISE will begin enrolling soon. PROMISE will evaluate the short- and long-term effects of triple-combination therapy on the overall health of people with CF, including infections, inflammation, mucus clearance, GI health, blood sugar, growth, and liver function. Talk to your care team about potentially participating in the PROMISE study.

Safety Profile and Interactions

Q6. Are there known risks or side effects? Is it safe?

- In clinical trials, Trikafta was shown to be safe and effective with potentially fewer negative side effects than previous modulators. Trikafta comes with warnings related to elevated liver function tests, drug-drug interactions with products that

are inducers or inhibitors of a certain liver enzyme, and the risk of cataracts in younger age groups.

Q7. Are there drug-drug interactions?

- Certain drugs may interact with Trikafta, including some antifungal medicines and some antibiotics.
- You should not take Trikafta if you are on certain antibiotics (rifampin or rifabutin), specific seizure medications, or St. John's Wort.
- Talk to your doctor about all your current medications before taking Trikafta.

Q8. Will I be able to stop my other medications and treatments?

- People with CF who take the drug will likely still need other daily treatments to manage the symptoms of the disease. This should be discussed with your care team before making any changes to your medications or treatments.
- We recognize that it is a burden to take daily medications. The Foundation is funding a study called SIMPLIFY that will look at whether stopping some medications for a few weeks impacts lung function in people with CF who are taking Trikafta.

Access and Coverage

Q9. How do I get Trikafta?

- If you think you may qualify, speak to your care team about being evaluated to see if your mutations qualify (remember, you only need ONE F508del) and whether you are a good candidate for the medicine.

Q10. Will my insurance cover Trikafta?

- Your coverage will depend on your insurance provider(s), and we expect policies to vary.

Q11. What is the Foundation doing to help people obtain coverage for this medication?

- The Foundation is proactively contacting major public and private insurers, including Medicaid programs and large national plans, to ensure they understand cystic fibrosis as well as the significance of Trikafta, and its potentially critical role in improving the lives of people with CF.
- We are working strategically with the community -- clinicians, patients, and families -- to urge payers to cover the drug as soon as possible.

Q12. Can I take Trikafta if I have received a lung transplant?

- Similar to other CFTR modulators, Trikafta is not expected to improve lung function or pulmonary symptoms following a lung transplant.
- Immunosuppressive drugs are required following transplantation. If Trikafta is considered, potential drug-drug interactions must be anticipated. Before making any changes in your treatment regimen, discuss it with your transplant team.

Q13. When will Trikafta be expanded to younger age groups?

- It is too early to know when the expansion to people under 12 years old may occur. A trial of Trikafta in children with CF ages 6 to 11 is already underway with results expected in 2020.

Q14. Will Trikafta eventually be approved for people with other mutations?

- The Foundation is funding lab research to test whether people with other rare mutations may benefit from Trikafta. If positive, Vertex will still need to apply to the FDA to include these mutations on the label.

Q15. What financial assistance is available for patients who cannot afford the out of pocket cost of Trikafta?

- *Compass*, a personalized service provided by the CF Foundation, is available to help people with CF navigate their insurance, coordinate and maximize benefits, and locate funds that might free up financial resources in other parts of life such as housing

or rent. *Compass* can also help identify national and local non-profit assistance foundations, as well as connect you with Vertex, to understand all copay and financial assistance opportunities available. To get started, call 844-COMPASS (844-266- 7277) Monday through Friday, 9 a.m. until 7 p.m. ET, or email compass@cff.org.

- Vertex Pharmaceuticals, the manufacturer of Trikafta, offers copay assistance for eligible individuals with commercial/private insurance. For more information on Vertex's GPS program, including eligibility requirements for assistance, contact Vertex at 1-877-752-5933 or visit www.VertexGPS.com.
- National and local non-profit patient assistance foundations, like the HealthWell Foundation, offer out-of-pocket cost assistance for Trikafta as well as other CF-specific medications. Because each program has specific enrollment criteria, it is important to contact them to determine your eligibility. For more information about HealthWell's CF Fund, contact (800) 675-8416.

Q16. What financial assistance is available for patients on Medicare or Medicaid?

- *Compass*, a personalized service provided by the CF Foundation, is available to help people with CF navigate their insurance, coordinate and maximize benefits, and locate funds that might free up financial resources in other parts of life such as housing or rent. *Compass* can also help identify national and local non-profit assistance foundations to understand all copay and financial assistance opportunities available. To get started, call 844-COMPASS (844-266- 7277) Monday through Friday, 9 a.m. until 7 p.m. ET, or email compass@cff.org.
- Although manufacturer programs, including Vertex GPS, do not provide copay assistance to people with public insurance, there are national and local non-profit foundations, like the HealthWell Foundation, that offer out-of-pocket cost assistance for Trikafta as well as other CF-specific medications. Because each program has specific enrollment criteria, it is important to contact them to determine your eligibility. For more information about HealthWell's CF Fund, contact (800) 675-8416.

Additional Information

Q17. What is the Foundation doing for people who will not benefit from Trikafta?

- We recognize that not all people with CF will benefit from the triple-combination therapy, and we are steadfast in our commitment to finding treatments for the underlying cause of the disease for all people with CF, including those with two nonsense or other rare mutations. Learn more about the potential treatments the Foundation is exploring for people with [nonsense and other rare mutations](#).
- We are also committed to developing better treatments for CF beyond fixing the broken CFTR protein, including better antibiotics, mucus medicines, enzymes and anti-inflammatories.