



Cystic Fibrosis Foundation
FAQ: ICER Review of CFTR Modulators
Updated: August 24, 2020

1. What is ICER and what is the goal of the assessment?

- The [Institute for Clinical and Economic Review](#) (ICER) is a nonprofit research institute that produces reports that aim to contribute to the overall understanding of the cost and value of prescription drugs and health care services. Their reports include information on how well therapies work, how they compare to available treatments, and the relative value to patients and the health care system overall.
- In May 2018, [ICER published an evidence report](#) analyzing the comparative clinical effectiveness and value of ivacaftor (Kalydeco®), lumacaftor/ivacaftor (Orkambi®), and tezacaftor/ivacaftor (Symdeko®). The report found that although the therapies are clinically significant, the prices would have to be cut between 40 and 70 percent to be cost-effective. You can view the CF Foundation's response to the 2018 assessment [here](#).
- In September 2019, [ICER initiated an assessment](#) of the comparative clinical effectiveness and value of cystic fibrosis modulators. In addition to reviewing Kalydeco, Orkambi, and Symdeko, the report will focus on elexacaftor/tezacaftor/ivacaftor (Trikafta®), the triple-combination therapy approved by the U.S. Food and Drug Administration in October 2019.
- [On April 27, 2020](#), ICER published their revised evidence report and response to public comments. ICER's report concluded that while Trikafta received ICER's highest rating on clinical effectiveness, the price of Trikafta would need to be cut by 73 percent to be cost-effective. The three additional modulators would have to be similarly discounted. Visit [ICER's website](#) to read the report and public comments.

2. How will the information in this report be used?

- The final ICER report, slated for release in September 23, will provide more context around how modulators are covered by insurers and future research needs.
- Cost-effectiveness analyses must be used carefully and as part of a comprehensive evaluation of the value a treatment provides. CFTR modulators have had a life-changing effect for many people with CF. ICER's model is limited and should not be the sole source for making decisions about coverage.

3. Will it impact the ability for me/my family to get the treatments covered by our insurance?

- For most people, the answer is no. ICER's reports have had limited impact on coverage of treatments for people with CF. However, payers and policymakers may reference the report to understand the impact and significance of CFTR modulators.

4. Has this kind of review happened when other CF therapies were approved?

- Yes. In May 2018, [ICER published an evidence report](#) analyzing the comparative clinical effectiveness and value of ivacaftor (Kalydeco®), lumacaftor/ivacaftor (Orkambi®), and



tezacaftor/ivacaftor (Symdeko®). The report found that although the therapies are clinically significant, the prices would have to be cut between 40 and 70 percent to be cost-effective.

5. Does the CF Foundation participate in this process?

- The CF Foundation is participating in this process to help ICER understand cystic fibrosis and the complexity of treating the disease. Our expertise and years conducting clinical research gives us a unique perspective to offer insights to the ICER team. We also connected ICER with people from our community to ensure their voices of people with CF were represented throughout the process.
- The CF Foundation is also committed to generating data that will advance understanding of the long-term benefits of modulator therapies and their impact on the overall CF care regimen. For example, researchers are designing a study, known as SIMPLIFY, that will assess which medications people with CF could potentially stop taking once they start on a modulator. Additionally, a new clinical study called PROMISE will evaluate the short- and long-term effects of Trikafta on the overall health of people with CF including infections, inflammation, mucus clearance, GI health, blood sugar, growth, and liver function.
- After postponing the public meeting due to the COVID-19 pandemic, originally scheduled for April 27, ICER hosted a virtual public meeting on August 27. During the meeting, ICER will report its findings and provide a forum for participation from patients and other stakeholders to share their experience with CFTR modulators. The CF Foundation will participate in this meeting alongside several members from the CF community.

6. Does the CF Foundation agree with the way in which ICER completes its assessment?

- While cost-effectiveness analyses can be informative, they must be used carefully and as part of a holistic evaluation of the value a treatment provides.
- The CF Foundation welcomes ICER's efforts to add more data and transparency to the important discussion around cost. However, we continue to have reservations about the model ICER used to generate their assessments and do not believe that their reports reflect the full value CFTR modulator therapies will provide over time. Important key points missing from ICER's report include:
 - i. **Early initiation and long-term use of these drugs will have profound implications in altering the course of the disease.** An early start on modulators could dramatically change standard of care and prevent a host of complications, such as serious lung damage, declining pancreatic function, and challenges with nutrient absorption and weight.
 - ii. **CFTR modulators have clinical and quality of life benefits beyond lung function.** Forced expiratory volume in one second (FEV1) is an important -- but not the only or best -- sign that a CFTR modulator is having a disease-modifying effect. Focusing on FEV1 undervalues and under-predicts the long-term, systemic benefits that people with CF derive from CFTR modulators.



- iii. **The societal benefits associated with modulators will be seen in time.** At best, the model captures a fraction of how modulators affect quality of life, education, and work productivity.

7. Did people with CF and their families participate in the process?

- Yes, ICER provided several public comment periods which generate input from people with CF and their families, patient advocacy organizations, and industry. In April, ICER [responded to public comments](#) as part of their revised evidence report.
- Additionally, members of the CF community will provide oral testimony during ICER's public meeting on August 27.

8. Does the Foundation agree with ICER's recommendation that modulators be discounted by more than 70 percent?

- The CF Foundation doesn't play a role in pricing decisions, but we do recognize that the cost of CF therapies can be a barrier to accessing care. People with CF who are eligible for modulator therapy must have access to the most effective therapy available to them. We believe that patients should not bear undue cost and coverage burdens to access life-changing drugs. That is why we are steadfast in our commitment to fight for adequate, affordable access to high-quality, specialized care.
- ICER's assessment does not fully capture the clinical importance of these drugs because we do not yet have comprehensive data on the long-term impact of these promising drugs.
- Starting a modulator at an early age may prevent serious lung damage and other manifestations of the disease.

9. What's a QALY and how is it used?

- A QALY, or quality-adjusted life-year, is used to measure how well a medical treatment improves patients' lives by looking at improvements in quality and length of life. Along with the academic community, drug manufacturers, and government agencies in the U.S. and globally, ICER uses the QALY as a core measure of how well different treatment options improve the quality and/or length of life for patients. ¹

10. I have more questions about the process. Who should I talk to?

- You can email the CF Foundation at publicpolicy@cff.org.

¹ <https://icer-review.org/announcements/icer-describes-qaly/>