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

   - The [Institute for Clinical and Economic Review](https://www.icer.org) (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](https://www.cff.org/research-news/).  

   - 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.

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

   - The ICER report is intended to provide more information to policymakers and insurers to help them make informed coverage decisions and to better understand the value of therapies. ICER does not have authority to shape public policy.

   - 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. Application of ICER’s model to inform real-world coverage decisions must recognize its flawed assumptions and limitations.

   - The CF Foundation believes that manufacturers and public and private payers must work together on solutions that provide access to lifechanging therapies.

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 report 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 that 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.
   - The CF Foundation had serious reservations about the model ICER used to generate the 2018 assessment, which did not capture important key points about modulators, including:
     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.

6. **Can people with CF and their families participate in the process?**
   - Yes, ICER provides several public comment periods which generate input from people with CF and their families, patient advocacy organizations, and industry.
   - The next comment period starts February 20 and ends March 18. For more information, visit [https://icer-review.org/topic/cystic-fibrosis/](https://icer-review.org/topic/cystic-fibrosis/).

7. **Does the Foundation agree with ICER’s recommendation that modulators be discounted by more than 50 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. 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 evidence on the long-term impact of these promising drugs.

• For people who start the drug at an early age, we believe the course of the disease could be altered – preventing serious lung damage and preserving pancreatic function.

8. **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.  

9. **I have more questions about the process. Who should I talk to?**
   • You can email the CF Foundation at publicpolicy@cff.org.

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