1. What is ICER and what is the goal of the report?
   - 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 specialty therapies. 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.
   - The ICER report on cystic fibrosis therapies assessed the clinical effectiveness and value of cystic fibrosis transmembrane conductance regulator (CFTR) modulators including ivacaftor (Kalydeco®), lumacaftor/ivacaftor (Orkambi®), and tezacaftor/ivacaftor (Symdeko™).

2. How will the information in this report be used?
   - Overall, policymakers and public and private insurers are looking for more information to help them make informed coverage decisions and to better understand the value of therapies.
   - 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 inaccuracies and limitations.

3. Will it impact the ability for me/my family to get the treatments covered by our insurance?
   - It’s important to remember that the ICER report focuses on CFTR modulator therapies, not all CF treatments. The recommendations within the report are nonbinding and will be considered one resource, among others, as public and private insurers make coverage decisions.

4. Has this kind of review happened when other CF therapies were approved?
   - No, this is the first ICER review of CF therapies. ICER began evaluating the cost effectiveness of drugs in 2014, and to date, has looked at treatments for diseases other than CF.

5. Does this impact the FDA’s approval process of the treatments?
   - No, the FDA reviews therapies to ensure that they are safe and effective.

6. Did the CF Foundation participate in this process?
   - The 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 the voices of people with CF were represented throughout the process.

- Our goal was for ICER’s report to accurately depict the needs and challenges of people living with the disease and how modulator therapies fit into the greater picture of care. However, we have serious reservations about the model used to generate this report and are concerned that it does not reflect the complexity of CF, the realities that patients experience, and the clinical significance of CFTR modulators.

7. Did people with CF participate in the process?

- Yes, the public comment period generated input from people with CF and their families, patient advocacy organizations, and industry.

- The CF Foundation connected ICER with people from our community to ensure the voices of people with CF were represented throughout the process.