



Modulator Treatments for Cystic Fibrosis: Effectiveness and Value

Draft Questions for Deliberation and Voting: April 30, 2020 Public Meeting

These questions are intended for the deliberation of the California Technology Assessment Forum (CTAF) voting body at the public meeting.

Treatments

- Trikafta™ (elexacaftor/tezacaftor/ivacaftor, Vertex Pharmaceuticals, Inc.)
- Orkambi® (lumacaftor/ivacaftor, Vertex Pharmaceuticals, Inc.)
- Symdeko® (tezacaftor/ivacaftor, Vertex Pharmaceuticals, Inc.)

Clinical Evidence

Patient Population 2 (Questions 1-2): Individuals with CF who are homozygous for the F508del mutation

1. Is the evidence adequate to demonstrate that the net health benefit of treatment with Trikafta with best supportive care is greater than that of best supportive care alone?

Yes No

If yes, answer question 2. If no, move to question 3

2. Is the evidence adequate to demonstrate that the net health benefit of treatment with Trikafta and best supportive care is greater than that of treatment with Symdeko and best supportive care?

Yes No

Patient Population 3 (Questions 3-4): Individuals with CF who are heterozygous for the F508del mutation with a residual function mutation.

3. Is the evidence adequate to demonstrate that the net health benefit of treatment with Trikafta with best supportive care is greater than that of best supportive care alone?

Yes No

If yes, answer question 4. If no, move to question 5

4. Is the evidence adequate to demonstrate that the net health benefit of treatment with Trikafta and best supportive care is greater than that of treatment with Symdeko and best supportive care?

Yes No

Patient Population 4: *Individuals with CF who are heterozygous for the F508del mutation with a minimal function mutation.*

5. Is the evidence adequate to demonstrate that the net health benefit of treatment with Trikafta with best supportive care is greater than that of best supportive care alone?

Yes No

Potential Other Benefits and Contextual Considerations

6. **When compared to best supportive care**, does treating patients with Trikafta offer one or more of the following potential “other benefits?” (select all that apply)
- a. This intervention offers reduced complexity that will significantly improve patient outcomes.
 - b. This intervention will reduce important health disparities across racial, ethnic, gender, socioeconomic, or regional categories.
 - c. This intervention will significantly reduce caregiver or broader family burden.
 - d. This intervention offers a novel mechanism of action or approach that will allow successful treatment of many patients for whom other available treatments have failed.
 - e. This intervention will have a significant impact on improving patients’ ability to return to work and/or their overall productivity.
 - f. This intervention will have a significant positive impact outside the family, including on schools and/or communities.
 - g. There are other important benefits or disadvantages that should have an important role in judgments of the value of this intervention: _____
7. Are any of the following contextual considerations important in assessing Trikafta’s long-term value for money? (select all that apply)
- a. This intervention is intended for the care of individuals with a condition of particularly high severity in terms of impact on length of life and/or quality of life.
 - b. This intervention is intended for the care of individuals with a condition that represents a particularly high lifetime burden of illness.
 - c. This intervention is the first to offer any improvement for patients with this condition.

- d. Compared to best supportive care, there is significant uncertainty about the long-term risk of serious side effects of this intervention.
- e. Compared to best supportive care, there is significant uncertainty about the magnitude or durability of the long-term benefits of this intervention.
- f. There are additional contextual considerations that should have an important role in judgments of the value of this intervention: _____.

Long-term Value for Money

Patient Population 2: *Individuals with CF who are homozygous for the F508del mutation*

- 8. Given the available evidence on comparative clinical effectiveness and incremental cost effectiveness, and considering other benefits and contextual considerations, what is the long-term value for money of Trikafta with best supportive care compared with best supportive care alone?
 - a. Low long-term value for money at current pricing
 - b. Intermediate long-term value for money at current pricing
 - c. High long-term value for money at current pricing

Patient Population 3: *Individuals with CF who are heterozygous for the F508del mutation with a residual function mutation.*

- 9. Given the available evidence on comparative clinical effectiveness and incremental cost effectiveness, and considering other benefits and contextual considerations, what is the long-term value for money of Trikafta with best supportive care compared with best supportive care alone?
 - a. Low long-term value for money at current pricing
 - b. Intermediate long-term value for money at current pricing
 - c. High long-term value for money at current pricing

Patient Population 4: *Individuals with CF who are heterozygous for the F508del mutation with a minimal function mutation.*

- 10. Given the available evidence on comparative clinical effectiveness and incremental cost effectiveness, and considering other benefits and contextual considerations, what is the long-term value for money of Trikafta with best supportive care compared with best supportive care alone?
 - a. Low long-term value for money at current pricing
 - b. Intermediate long-term value for money at current pricing
 - c. High long-term value for money at current pricing