



Disease Modifying Therapies for Treatment of Transthyretin Amyloid Cardiomyopathy (ATTR-CM)

Draft Questions for Deliberation and Voting: September 20th, 2024 Public Meeting

These questions are intended for the deliberation of the Midwest CEPAC voting body at the public meeting.

Patient Population for all questions: Adults with transthyretin amyloid cardiomyopathy (ATTR-CM).

Clinical Evidence

1. For adults with ATTR-CM is the current evidence adequate to demonstrate that the net health benefit of **tafamidis** is greater than that of **no disease-specific treatment**?

Yes No

2. For adults with ATTR-CM is the current evidence adequate to demonstrate that the net health benefit of **acoramidis** is greater than that of **no disease-specific treatment**?

Yes No

3. For adults with ATTR-CM is the current evidence adequate to demonstrate that the net health benefit of **vutrisiran** is greater than that of **no disease-specific treatment**?

Yes No

4. For adults with ATTR-CM is the current evidence adequate to demonstrate that the net health benefit of **vutrisiran added to tafamidis** is greater than that of **tafamidis alone**?

Yes No

5. Is the currently available evidence adequate to distinguish the net health benefit among the **interventions when used as monotherapy (tafamidis, acoramidis, vutrisiran)**?

Yes No

5a. If "Yes", which therapy has the greatest net health benefit?

Benefits Beyond Health and Special Ethical Priorities

To help inform judgments of overall long-term value for money, please indicate your level of agreement with the following statements:

1=Strongly Disagree; 2=Disagree; 3=Neutral; 4=Agree; 5=Strongly Agree

6. There is substantial unmet need despite currently available treatments.
7. This condition is of substantial relevance for people from a racial/ethnic group that have not been equitably served by the healthcare system.

The following questions are about the specific treatments:

1=Strongly Disagree; 2=Disagree; 3=Neutral; 4=Agree; 5=Strongly Agree

8. The TTR stabilizers are likely to produce substantial improvement in caregivers' quality of life and/or ability to pursue their own education, work, and family life.
9. Acoramidis offers a substantial opportunity to improve access to effective treatment by means of its mechanism of action or method of delivery.

Long-Term Value for Money

10. Given the available evidence on comparative clinical effectiveness and incremental cost effectiveness, and considering benefits beyond health and special ethical priorities, what is the long-term value for money of **tafamidis compared to no disease-specific treatment** at current pricing?
 - a. High long-term value for money at current pricing
 - b. Intermediate long-term value for money at current pricing
 - c. Low long-term value for money at current pricing
11. Given the available evidence on comparative clinical effectiveness and incremental cost effectiveness, and considering benefits beyond health and special ethical priorities, what is the long-term value for money of **acoramidis compared to no disease-specific treatment** at assumed pricing?*

 - a. High long-term value for money at assumed pricing
 - b. Intermediate long-term value for money at assumed pricing
 - c. Low long-term value for money at assumed pricing

**This vote will only be taken if a reliable estimate of the price becomes available for acoramidis.*