Mavacamten for Hypertrophic Cardiomyopathy

Revised Questions for Deliberation and Voting: October 22, 2021 Public Meeting

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

Patient Population for all questions: Adults with symptomatic hypertrophic obstructive cardiomyopathy (HOCM) on background therapy with beta blockers and/or calcium channel blockers.

Clinical Evidence

1. Is the currently available evidence adequate to demonstrate that the net health benefit of mavacamten added to background therapy is superior to that provided by background therapy alone?
   
   Yes | No

2. Is the currently available evidence adequate to demonstrate that the net health benefit of mavacamten is superior to that provided by disopyramide?
   
   Yes | No

Contextual Considerations and Potential Other Benefits or Disadvantages

Please vote on the following contextual considerations:

When making judgments of overall long-term value for money, what is the relative priority that should be given to any effective treatment for HOCM on the basis of the following contextual considerations:

1=Very low priority; 2=Low priority; 3=Average priority; 4=High priority; 5=Very high priority

3. Acuity of need for treatment of individual patients based on the short-term risk of death or progression to permanent disability
4. Magnitude of the lifetime impact on individual patients of the condition being treated
5. Other (as relevant)
Please vote on the following potential other benefits or disadvantages:

What are the effects of mavacamten on the following outcomes that inform judgment of the overall long-term value for money of mavacamten?

1=Major negative effect; 2=Minor negative effect; 3=No difference; 4=Minor positive effect; 5=Major positive effect

6. Patients’ ability to achieve major life goals related to education, work, or family life
7. Caregivers’ quality of life and/or ability to achieve major life goals related to education, work, or family life
8. Patients’ ability to manage and sustain treatment given the complexity of regimen
9. Society’s goal of reducing health inequities
10. Opportunity to improve access to treatment
11. Availability of a treatment with different timing and types of risks and benefits, relative to existing procedural and surgical options
12. Other (as relevant)

Long-Term Value for Money

Value votes will not be taken at the October 22, 2021 Public Meeting because a net price for mavacamten is not currently available.