



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 distinguish the net health benefit between mavacamten and disopyramide?

Yes No

- a. **If the answer to Q2 is Yes:** Based on the available evidence, which therapy has a greater net health benefit: a) mavacamten, or b) disopyramide?

a) Mavacamten b) Disopyramide

3. Is the currently available evidence adequate to distinguish the net health benefit between mavacamten and septal reduction therapies (i.e., myectomy and septal ablation)?

Yes No

- b. **If the answer to Q3 is Yes:** Based on the available evidence, which therapy has a greater net health benefit: a) mavacamten, or b) septal reduction therapies?

a) Mavacamten b) Septal reduction therapies

Contextual Considerations and Potential Other Benefits or Disadvantages

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

4. Acuity of need for treatment of individual patients based on the short-term risk of death or progression to permanent disability
5. Magnitude of the lifetime impact on individual patients of the condition being treated
6. Other (as relevant)

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

7. Patients' ability to achieve major life goals related to education, work, or family life
8. Caregivers' quality of life and/or ability to achieve major life goals related to education, work, or family life
9. Patients' ability to manage and sustain treatment given the complexity of regimen
10. Society's goal of reducing health inequities
11. Other (as relevant)

Long-Term Value for Money¹

12. Given the available evidence on comparative effectiveness and incremental cost-effectiveness, and considering other benefits, disadvantages, and contextual considerations, what is the long-term value for money of treatment with mavacamten added to background therapy versus background therapy alone?
 - a. Low long-term value for money
 - b. Intermediate long-term value for money
 - c. High long-term value for money

13. Given the available evidence on comparative effectiveness and incremental cost-effectiveness, and considering other benefits, disadvantages, and contextual considerations, what is the long-term value for money of treatment with mavacamten versus disopyramide?
 - a. Low long-term value for money
 - b. Intermediate long-term value for money
 - c. High long-term value for money

14. Given the available evidence on comparative effectiveness and incremental cost-effectiveness, and considering other benefits, disadvantages, and contextual considerations, what is the long-term value for money of treatment with mavacamten versus septal reduction therapies?
 - a. Low long-term value for money
 - b. Intermediate long-term value for money
 - c. High long-term value for money

¹ CTAF will vote on these questions if there is a net price available for mavacamten by the time of the public meeting.