



April 29, 2021

Institute for Clinical and Economic Review

2 Liberty Square, 9th Floor

Boston, MA 02109

Submitted Electronically: publiccomments@icer-review.org

Subject: Public Comments on Draft Scoping Document – Mavacamten

Dear Review Committee,

Thank you for the opportunity to provide feedback to ICER on its draft scoping document for comparative clinical effectiveness and value of mavacamten for hypertrophic cardiomyopathy. We appreciate your willingness to review comments and recommendations from the National Forum's Value & Access Steering Committee and partners working on these issues.

The Value & Access Steering Committee and partners jointly offer the following feedback for ICER's consideration in the development of the revised scoping document.

Recommendations:

Population:

- Focus on patients for whom there are data. The population studied in clinical trials were symptomatic HOCM patients who remained symptomatic despite treatment.

Outcomes:

- Revise “exercise restriction” to “exertional tolerance” or “capacity”
- Include measures that matter to HOCM patients, such as the characteristics captured in quality-of-life assessments such as the Kansas City Cardiomyopathy Questionnaire (KCCQ) or the Minnesota Living with Heart Failure Questionnaire.
 - Additional key symptoms and HOCM patient impacts¹, such as:
 - Tiredness
 - Shortness of breath with physical activity
 - Dizziness/light-headedness
 - Chest pain
 - Chest pain with physical exertion
 - Palpitations
 - Limitations on physical activities
 - Emotional impacts (anxiety, depression)
 - Impacts on work
- Evaluate lifestyle factors:

- HOCM is a condition that affects every patient differently. Due to this variability, we suggest looking as broadly as possible at lifestyle factors, including those highlighted in “The Voice of the Patient Report for Hypertrophic Cardiomyopathy (HCM): Proceedings from an Externally Led Public Patient-Focused Drug Development (PFDD) Meeting Corresponding to the FDA’s Patient-Focused Drug Development Meeting.”²
- Include measures such as:
 - Drop in gradient across the left ventricular outflow tract
 - Improvement in shortness of breath
 - Improvement in New York Heart Association (NYHA) class
- For future consideration:
 - Once properly diagnosed, patients with HOCM face life-long management of the condition. As patients are being identified earlier and younger, we suggest looking at evidence examining the impact on children and teens with HOCM.

Again, thank you for your consideration. We look forward to reviewing and providing additional comments throughout the review process.

Sincerely,

Members of the Value & Access Steering Committee and Partners representing the following organizations:

National Forum for Heart Disease & Stroke Prevention (convener)
American Association of Heart Failure Nurses
American College of Cardiology
American Heart Association
American Pharmacists Association Foundation
American Society for Preventive Cardiology
Association of Black Cardiologists
Association of State and Territorial Health Officials
BallengeRx Consulting
Global Healthy Living Foundation
Hypertrophic Cardiomyopathy Association
Independent Health
Institute for Patient Access
Mended Hearts
National Alliance of Healthcare Purchaser Coalitions
Partnership to Advance Cardiovascular Health
Partnership to Improve Patient Care
Preventive Cardiovascular Nurses Association
University of Michigan Center for Value-Based Insurance Design
WomenHeart

¹ Patient experiences with hypertrophic cardiomyopathy: a conceptual model of symptoms and impacts on quality of life. Zaiser E, Sehnert AJ, Duenas A, Saberi S, Brookes E, Reaney M. *J Patient Rep Outcomes*. 2020 Dec 1;4(1):102. doi: 10.1186/s41687-020-00269-8.

² Hypertrophic Cardiomyopathy Association. 2020. Voice of the Patient Report. <https://www.4hcm.org/finalreportpfdd>