Press Release
Perspectives of Patients and Providers on ICER's Assessment of Treatment for HCM
November 17, 2021

On November 16, 2021, the Institute for Clinical and Economic Review (ICER) published its final evidence report for mavacamten, a new treatment for Hypertrophic Cardiomyopathy (HCM), a disease of the heart muscle affecting over 1 in 500 people. HCM is a chronic disease that tends to become worse over time leading to lower quality of life for patients and long-term complications. HCM is also the most common reason for sudden cardiac death in adults under 35. There are currently no disease-specific medications for HCM and treatment often focuses on symptom management.

ICER conducted its assessment prior to having data from long-term studies into the effectiveness of the treatment, which was released last week. The FDA is also not scheduled to make a decision regarding approval of the first in class treatment until 2022.

Lisa Salberg, Founder and CEO of the Hypertrophic Cardiomyopathy Association (HCMA), engaged with ICER as a patient advocacy representative and patient throughout this process. Ms. Salberg began on the journey to educate, advocate, and support other patients based on her personal experience with HCM. This included medical errors that nearly cost her life, the death of her sister - also due to medical errors - and the diagnosis of several family members. The HCMA currently has 43 Recognized Center of Excellence programs for the treatment of HCM and represents over 15,000 families living with the condition.

Ms. Salberg stated in response to the final evidence report, "I question the value to patients as to why ICER assesses a treatment's effectiveness for payers before the FDA has even completed its own approval process. It was not easy to be an engaged patient in this process due to the short timeline and lack of investment by ICER in working with patient groups to harness patient data effectively. I do appreciate that ICER listened to the perspectives shared by myself, patients and clinical experts and incorporated some of our suggestions into its policy recommendations, most significantly the recommendation for mavacamten to be prescribed only by high volume HCM programs. Simultaneously, I worry that ICER's step therapy recommendation may cause payers to restrict access to this first-in-class treatment. Because patients like me were given a voice, the voting panel recognized the magnitude of the lifetime impact on individuals living with HCM as an important contextual consideration for any effective therapy for HCM. Although in the minority, 40% of the voting panel gave weight to the evidence's potential to adequately demonstrate net health benefit to patients of mavacamten when added to background therapy. Payers should reach out to HCMA if they want to truly understand this largely misunderstood condition and the impact of treatment as they make decisions about treatment coverage."

John Clymer, Executive Director of the National Forum for Heart Disease & Stroke Prevention, which convenes stakeholders including patient, provider, payer, and public health organizations to provide consensus feedback to ICER on its reviews of cardiovascular therapy, commended ICER for obtaining more patient input for this review. He then questioned the seeming dissonance between what was presented at the October 22 meeting of the appraisal committee and the final report, asking, "Is the voting panel punching holes in ICER's evidence review? Are they out of sync? ICER's evidence summary said, 'relative to usual care, mavacamten improves patient reported outcome and clinician-estimated function status. The voting panel said, the evidence is not adequate to demonstrate a net health benefit of mavacamten added to background therapy when compared to background therapy alone.

Is there enough strong evidence to draw conclusions? That appears to be in question. If not, then how much weight should payers, clinicians, and patients put in ICER's cost benchmarks and budget modeling for this particular therapy?"

Mr. Clymer advocated for across-the-board transparency in ICER's methodology to increase the value of its recommendations. He said, "Some of ICER's modeling assumptions are more transparent than others. The Community Preventive Services Task Force sets a good, high standard for transparency with the published methodology for its systematic reviews of evidence which are the gold standard in public health."

PIPC shares the concerns of the Hypertrophic Cardiomyopathy Association and the National Forum for Heart Disease & Stroke Prevention about the potential response from payers and the ultimate impact on patient access to care when considering the ICER report. Because patients like Lisa Salberg pushed so hard to be heard, the contextual considerations of the report were edited to better articulate the health benefit of treatment and the magnitude of lifetime impact of HCM, but those real-world considerations are too often lost when payers are more focused on the policy recommendations that justify coverage restrictions. The National Forum's efforts to develop consensus recommendations throughout the ICER review process raised serious methodological concerns. Going forward, we hope ICER will consider changes to its process to facilitate patient engagement and we urge payers to look beyond the ICER press release and instead take the time to directly engage with these patients and clinicians to understand their nuanced perspectives.