These comments are submitted in response to ICER's invitation to comment as a patient advocate and former participant of the ICER Public Policy discussion regarding mavacamten, a myosin inhibitor for the treatment of obstructive hypertrophic cardiomyopathy (HOCM).

I am Gwen Mayes, a 66 year old woman living with symptomatic HOCM since birth. I have been medically managed at centers of excellence and community cardiologists. For many years, I have been evaluated for a septal myectomy due to persistent symptoms (e.g., fatigue, SOB, syncope, anxiety, palpitations) and opted to wait and pursue treatment with mavacamten.

Clinical Impact I began daily use of Camzyos® 5 mg on July 7, 2022 and enrolled in the REMS program. My LVOT was 57 mmHg and EF 65%. Within *a few days,* I noticed less chest tightness, walked comfortably in my neighborhood, completed advanced Pilates classes, and had fewer palpitations. I had a positive, immediate response to Camzyos®: my LVOT dropped to 34 mg (4 weeks); 33 mg (8 weeks); and "single digits" (12 weeks) by September 29, 2022. My EF remained 65%. I had no noticeable heart murmur, was walking 3-4 miles/day, sailed in New England, carried backpacks through major airports, traveled internationally, and hiked trails on remote islands. I felt completely "normal" and fully alive for the first time in my life.

In early October, I went into atrial fibrillation which triggered events that continue to compromise my health now four months later. Since then, I have been an inpatient at 5 medical centers (2 COEs), hospitalized 14 days, had 7 seven medication changes, 9 cardioversions, been wheelchair bound, experienced 'early heart failure', and had a 9-hour ablation with no success. Although I was closely monitored and the algorithm for dosing was followed to a tee, it is thought the Camzyos® dosage should have been reduced to 2.5 mg (current dose) when my LVOT approached zero. Atrial fibrillation in HOCM patients is common; I have had episodes for three years. Yet little is known about the interaction of Camzyos® on the *entire* heart; clearly the drug does not work to inhibit myosin selectively. I suggest research on the effectiveness of the drug in patients by length of symptoms; the drug interface with atrial fibrillation management or any dysrhythmia; and the impact of the drug on 'normal' heart function.

REMS/Access- Frequent interactions with BMS nurse navigators for REMS compliance, e.g., echo appointments, added another level of scrutiny, repetitive reporting, and scheduling that created additional work. Their role needs evaluation. Further, access to the drug was impossible when traveling away from home. Once, while hospitalized my specialty pharmacy declined to refill the drug ("too early") necessitating my having to get a neighbor to my home, $100 FEDEX bill, hotel carrier to the hospital, etc. This inflexibility is scary and needs to be addressed. Similarly, changing dosage amount on short notice is not possible and this created uncertainty in my care on numerous occasions.

Gwen Mayes
HOCM Patient
1/4/2023