AfPA | Alliance for Patient Access

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# Optimizing Care for Hypertrophic Cardiomyopathy

A White Paper from the HCM Initiative of AfPA's Cardiovascular Disease Working Group


# Introduction

**As many as one in 250 people have hypertrophic cardiomyopathy.**[1] Also known as HCM, this condition causes the heart muscle to thicken, making it harder for the heart to pump blood.[2]

The heart has two key elements to pumping. The heart squeezes (systole) and relaxes (diastole). Because the thickness of the heart muscle makes it harder for the heart to relax, HCM is primarily a diastolic function disease.

An estimated 700,000 adults in the United States have HCM. Due to lack of awareness and challenges to accurate diagnosis, however, 85% remain undiagnosed and may be at significant risk.[3]

Among patients who are diagnosed with HCM, more than one-third require at least one hospitalization within the first year of diagnosis.[4]

Innovative treatments can help reduce the risk of hospitalization, minimize symptoms and improve health outcomes. But while these treatments can be life changing, patients can't always access them.

# About Hypertrophic Cardiomyopathy

Hypertrophic cardiomyopathy is a genetic myocardial disorder; it is a serious condition that can swiftly become critical as the disease progresses.

**Patients may experience a variety of symptoms:**[5]

- **Cardiac arrest**
- **Light-headedness**
- **Chest pain**
- **Palpitations**
- **Dizziness**
- **Shortness of breath**
- **Fainting**
- **Stroke**
- **Fatigue**

Some patients have a specific type of HCM known as obstructive hypertrophic cardiomyopathy. This occurs when the thickened muscle squeezes so that the walls of the heart come together and touch, preventing blood from flowing out of the heart and to the rest of the body. Patients often experience more symptoms with increasing severity.

Hypertrophic cardiomyopathy is common. Broader testing can help patients and health care providers identify the condition early and begin treating it.

The condition places a heavy burden on patients. Symptoms can be difficult to manage, and the disease does not yet have a cure. Disease management strategies have historically relied upon treating symptoms. But innovative, FDA-approved treatment has now opened doors for HCM patients.

# Treatment

Treatment for HCM has historically focused on alleviating symptoms rather than directly addressing the disease's underlying cause. Fortunately, new medications and surgical options have emerged.

## Treatment with Medication

The most common avenue of treatment is medication. Prescription drugs, including beta blockers, blood thinners, anti-arrhythmic medications and calcium channel blockers, reduce the risks of symptoms such as blood clots and strokes.

More than 75% of patients treated with cardiac myosin inhibitors see their symptoms improve, with sustained benefits over 120 weeks, research shows.[6,7]

Cardiac myosin inhibitors allow patients and providers to tailor treatment to individual needs and preferences—provided they are accessible.

## Cardiac Myosin Inhibitors

There is now a disease-specific medication to treat HCM. It directly treats the cause of the disease instead of its symptoms. Mavacamten, a type of medication known as a cardiac myosin inhibitor, was approved by the FDA in 2022 to treat the obstructive form of HCM. These oral treatments strengthen blood flow from the heart by relaxing the heart's contractions.

## Surgical and Catheter-Based Treatments

Surgical or catheter-based procedures, such as removing or ablating portions of the thickened heart muscle or implanting devices like pacemakers, can address HCM. These treatment options, however, come with risks, including infection, cardiac arrest or death. If a patient can achieve similar success with medications like cardiac myosin inhibitors, these risks can be avoided.

## Treating Hypertrophic Cardiomyopathy & Mental Health

Patients with health conditions like HCM may also have a mental health condition. Serious physical illnesses can result in new mental health challenges or exacerbate preexisting ones. To best care for patients, providers must make sure to provide support across all physical and mental conditions.

# Treatment Access Challenges

## Cardiac myosin inhibitors are exciting breakthrough treatment options for patients.

Many patients, unfortunately, face challenges getting access to these treatments and effectively managing their condition. Those challenges take several forms.

### Physician Availability & Knowledge Gaps

To get the right treatment, patients first need an accurate diagnosis. That can be difficult. Hypertrophic cardiomyopathy has become more well known in recent years, but some providers still lack awareness of the condition. Patients may not be connected to the right specialist in a timely manner, undermining their ability to access treatment.

Greater awareness would improve the speed of diagnosis and allow patients to be connected to an optimal treatment faster.

Genetic testing also plays an important role in diagnosing and treating HCM, for both patients and their families. Genetic counselors are specially trained to identify appropriate genetic testing, provide patient education, obtain informed consent and interpret genetic test results.

Accessing a specialist can be challenging, however. Limited insurance coverage of genetic counseling visits and genetic testing can present a barrier to equitable care for patients and their at-risk relatives. Without someone to interpret the results of a genetic test, patients may face delays in diagnosis and treatment.

## Health Insurance Barriers

One of the toughest barriers for patients is utilization management. These tactics are employed by health plans when determining if a patient will receive coverage for their prescribed treatment. They often delay or deny care, preventing patients from accessing the treatments they need.

### Prior Authorization

Prior authorization is one way that insurance companies delay or deny care. This happens when health plans must approve a clinician-prescribed medication before they cover it. The process often causes delays and can still end with patients being denied access.

As patients wait for approval, they may experience worsening symptoms and disease progression.

Prior authorization is one of most debilitating hurdles that patients and providers face. Patients with hypertrophic cardiomyopathy don't have time to waste waiting for approval. They need treatment swiftly upon diagnosis, and prior authorization prevents that.



### Step Therapy

Health plans also delay care through what's known as step therapy. Insurers may require patients to try and fail on lower-cost treatments before the plan will cover their prescribed treatment.

For a patient with a serious heart condition like HCM, time spent failing other treatments may allow for disease progression and worsening symptoms. It can even be fatal.

### Non-Medical Switching

Another common insurer tactic is non-medical switching. Health plans may move a patient's medication to a higher level on the formulary – increasing the copay and pricing the patient out of access – or simply eliminate coverage for the drug altogether. Patients who are stable on their medication are forced onto alternative treatments for non-medical reasons, simply because a different drug is more profitable for the health plan.

Non-medical switching can leave patients who were previously stable to face worsening symptoms and new side effects.

## New-to-Market Exclusions

Patients may have to wait years before an approved medication is actually available to them. Health plans may not add a new medication to their formulary, delaying access to innovative options. Some insurers update their formularies only once a year. Others may wait for a treatment to be on the market for several years before considering it. Arbitrary delays may keep an innovative treatment from a patient who urgently needs it.

## Copay Accumulator Adjustment Programs

Copay accumulator adjustment programs also pose a serious challenge to patient access.

Patients may rely upon copay assistance to afford their medication. When health plans institute copay accumulator adjustment programs, however, patients' copay assistance no longer counts toward their deductible. Patients may then face significant out-of-pocket costs once their copay assistance has been used up. Many patients may stop taking their medication because they can no longer afford it. Without treatment, patients' conditions will continue to worsen, and they may lose the progress they've made.

## Imaging Barriers

The FDA mandates a Risk Evaluation and Mitigation Strategy, or REMS, for cardiac myosin inhibitors, which includes regular echocardiograms for patients. They must be monthly for the first three months. Then, patients should receive an echocardiogram every three months.

Hypertrophic cardiomyopathy can mimic more common heart conditions, making it difficult to diagnose without these screenings. Echocardiograms may be needed regularly after diagnosis to monitor for complications. But some insurers regard monthly imaging as excessive and unnecessary, leading to extra reauthorization procedures or authorization denials.

## Specialty Pharmacies

New or complex medications, like those for HCM, often need special handling or storage. Such accommodations aren't necessarily common in standard pharmacy chains. Patients must turn to specialty pharmacies to access their treatments. But these facilities are often unfamiliar to many patients, which can be disorienting and frustrating.

Patients need education and information about specialty pharmacies so they can swiftly access their treatment.

# Advocating for Patient Access

**Patients with hypertrophic cardiomyopathy face numerous hurdles.**
**Good policies, however, can help.**

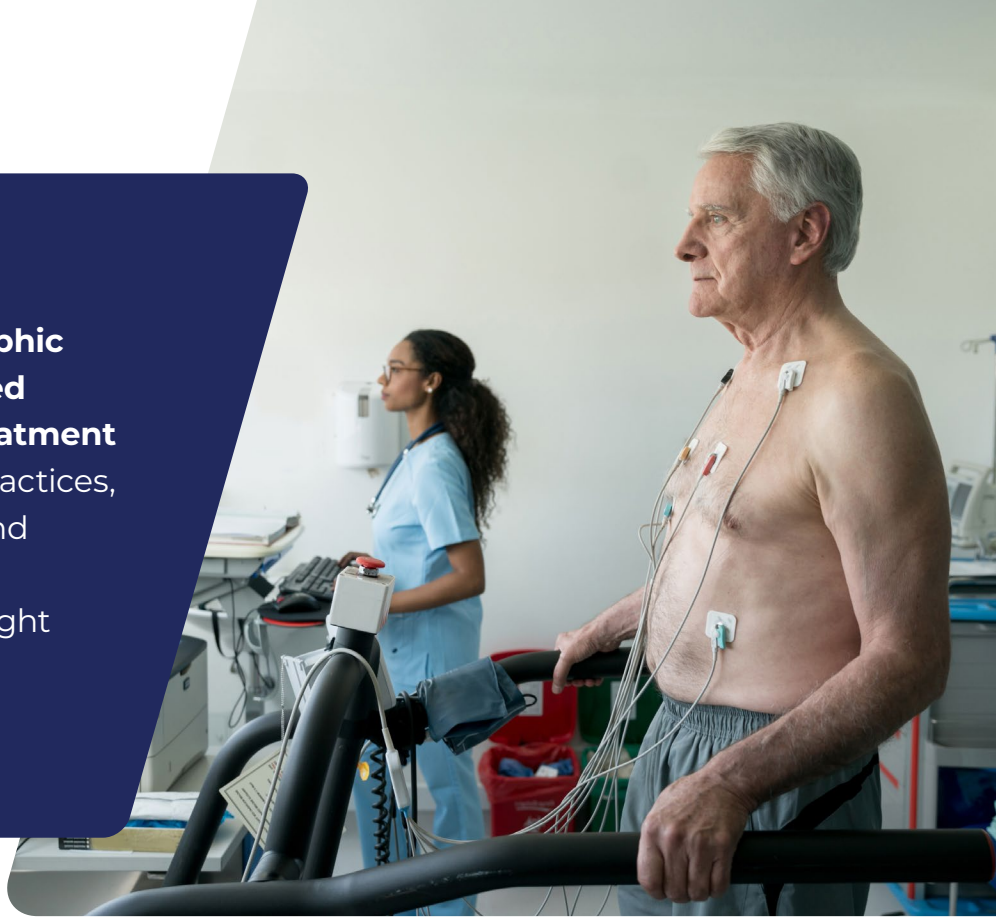
Policymakers can reduce access barriers for patients and their providers by:

- **Raising awareness & increasing education**
- **Minimizing health plan interference & limiting utilization management**
- **Protecting patients from imaging restrictions**

With the right support, patients with HCM can access the treatment necessary to best manage their condition.

# Conclusion

**To optimize care for hypertrophic cardiomyopathy, patients need access to the full range of treatment options.** Implementing best practices, improving patient education and enhancing collaboration can all enable patients to access the right treatment and pursue better health outcomes.



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The Alliance for Patient Access is a national network of policy-minded health care providers advocating for patient-centered care.

**AllianceforPatientAccess.org**


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