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Drug trials of new hypertrophic cardiomyopathy treatment brings hope to patients

Hypertrophic cardiomyopathy is a condition where the heart muscle walls are thickened. This interferes with the heart's ability to pump blood around the body. Current medication focuses on hypertrophic cardiomyopathy symptom management, but a new drug that may treat the underlying cause is under review.

Hypertrophic cardiomyopathy, a condition affecting the heart muscle, affects around 1 in 500 people. While many people can manage their symptoms through healthy lifestyle habits, for others symptoms can worsen over time.

These people have a greater risk of developing other health complications as well as sudden cardiac death, and death from heart failure. Fortunately, mortality rates from hypertrophic cardiomyopathy are quite low - about 1% of cases per year.

Hypertrophic cardiomyopathy treatment currently focuses on symptom relief. However, drug trials suggest that a new drug may be able to address the underlying cause of the condition.

What is hypertrophic cardiomyopathy?

People with hypertrophic cardiomyopathy have a thickened heart muscle. Usually, the condition is caused by abnormal genes that cause the walls of the left ventricle (heart chamber) to contract harder and become thicker. This affects the heart's ability to pump blood to the rest of your body because:

- The heart muscle may be stiffer, which means it can't take in as much blood between beats.

- The flow of blood into your aorta (the biggest artery in the body) may be obstructed.
- Your heart valves may become leaky, allowing blood to flow backwards and reducing the efficiency of filling of the heart chambers.

Most people have no symptoms and continue to lead normal lives. They may be advised to manage their disease by implementing lifestyle changes that reduce strain on the heart and prevent damage. These can help to lower the risk of the condition getting worse and resulting in other health complications.

Lifestyle changes that may help manage hypertrophic cardiomyopathy

- Reaching or achieving a healthy weight.
- Limiting alcohol consumption.
- Quitting smoking.
- Refraining from strenuous exercise, if advised.

Symptoms of hypertrophic cardiomyopathy

- Shortness of breath.
- Chest pain (angina).
- Palpitations.
- Dizziness and fainting attacks.

For those who do have symptoms, they can range in severity and may not develop straightaway.

Existing hypertrophic cardiomyopathy treatment for symptoms

Currently, hypertrophic cardiomyopathy treatment includes medications that can ease these symptoms. These medicines can improve a person's quality of life.

These include:

- Beta-blockers - slow the heart rate and make the heart contract less forcefully, which allows more time for the ventricle to fill with more blood each heartbeat.
- Anti-arrhythmic medicines - treat and prevent abnormal heart rhythms (arrhythmias) by helping to correct the electrical impulses in the heart.
- Anticoagulant medicine - if you develop atrial fibrillation (the most common arrhythmia in the UK) this medication thins the blood to help prevent blood clotting.

In severe cases, surgery may be an option. Types of procedures include:

- An operation to remove a segment of thickened muscle (myectomy).
- Alcohol injection into the small blood vessels to destroy muscle (alcohol septal ablation).
- Valve replacement.
- Heart transplant (very rarely).

Possible complications

For many people taking hypertrophic cardiomyopathy treatment medicines, quality of life can greatly improve. This is because once a person stops growing, the thickening of the heart muscle often stops and remains stable.

However, others find that as their heart muscle stiffens their condition can get progressively worse and it may cause other health problems.

Health complications of hypertrophic cardiomyopathy include:

- Arrhythmias - abnormal heart rhythms.
- Infective endocarditis.
- Sudden death - this is rare and probably due to a severe and sudden onset of arrhythmia.

Drug trials of mavacamten

Clinical trials of a new medicine called mavacamten have shown promising results, both in terms of symptom improvement and in treating the underlying cause of the condition. This new drug is currently undergoing review before its effectiveness and safety are approved.

If mavacamten is approved by the drug regulators on the basis of these studies, it will be the first drug on offer that improves the condition itself. This is an exciting prospect that brings hope to those living with the fear that their hypertrophic cardiomyopathy may progress and cause potentially life-threatening complications.

Phase 3 EXPLORER-HCM trial - 'A Randomized, Double Blind, Placebo Controlled Clinical Study to Evaluate Mavacamten in Adults With Symptomatic Obstructive Hypertrophic Cardiomyopathy'.
This study took place at 68 clinical cardiovascular centres in 13 countries. 251 patients over 18 years of age were randomly assigned either mavacamten or a placebo drug for 30 weeks, followed by an 8-week washout period.

Drug trial results

Compared to patients in the placebo arm, patients treated with mavacamten were more likely to show the following:

Improvement of symptoms

- Improved shortness of breath by increasing oxygen intake.
- Improved symptoms of heart failure.
- Improved exercise capacity.
- Patients felt that they could participate in daily activities more easily.

Treatment of underlying cause

The new drug appears to improve heart structure abnormalities:

- Decreases stiffness of the heart muscle wall.
- Restores normal valve movements (reduces the intensity of contractions).

Limitations of drug trials

- 28% of patients were missing either baseline or follow-up data.
- Only symptomatic patients were included - additional data are needed to confirm comparable benefits for asymptomatic patients, in terms of the drug's ability to treat the underlying cause.
- Longer-term studies are needed to understand longer-term outcomes.
- Benefits regress with treatment withdrawal.

How does mavacamten work?

Mavacamten reduces the thickness of the heart muscle walls and improves blood flow through the heart. The drug targets a protein responsible for the contractions of the heart muscle. In hypertrophic cardiomyopathy patients, the thickened heart muscle walls contract harder than they should. This drug works by reducing the intensity of these contractions, allowing more blood to be pumped in and out of the heart.

How could this hypertrophic cardiomyopathy treatment benefit patients?

The drug trials suggest that mavacamten can improve health status in patients with symptomatic hypertrophic cardiomyopathy, including improvements to blood flow, symptoms, well-being and ability to participate in daily activities. The medicine appears to work quickly, as these changes were observed early after treatment.

More data are needed to determine the long-term outcomes of this medicine's ability to treat the underlying cause of the condition. This does, however, provide some hope that in future we may have a treatment that can help to halt the progression of hypertrophic cardiomyopathy in some people. A reduction in the risk of other health complications could improve mortality rates.

What are the next steps?

For now, analysis of data is ongoing. On 18 November 2021, the US Food and Drug Administration (FDA) extended the review of this hypertrophic cardiomyopathy treatment until April 2022. While no new studies have been requested, the risk evaluation strategy remains under review.

Bristol Myers Squibb, who developed the new drug, remain confident in mavacamten: "This first-in-class cardiac myosin inhibitor demonstrated clinically meaningful improvements in symptoms, functional status, and quality of life," says Samit Hirawat, executive vice president and chief medical officer of global drug development.

"We look forward to continuing to work closely with the FDA to bring this important medicine to patients."

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