NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Health Technology Evaluation

Aficamten for treating symptomatic obstructive hypertrophic cardiomyopathy ID6575

Draft scope

Draft remit/evaluation objective

To appraise the clinical and cost effectiveness of aficamten within its marketing authorisation for treating symptomatic obstructive hypertrophic cardiomyopathy.

Background

Hypertrophic cardiomyopathy (HCM) is a genetic condition that is most often caused by a change or fault (or mutation) in one or more genes and is characterised by the thickening of the muscular wall of the heart (the myocardium). Thickening of the septum (the dividing wall between the left and the right side of the heart), resulting in reduced or restricted blood flow is classified as obstructive HCM. Most people with HCM may initially have few or no symptoms. However, the disease is progressive, and symptoms may develop or worsen at any age. Common symptoms of HCM include shortness of breath, chest pain, palpitations, light headedness, and fainting[1]. People with obstructive HCM can develop serious complications such as atrial fibrillation, heart failure, malignant ventricular arrhythmias, and sudden cardiac death[2].

HCM is the most common genetic cardiovascular disease and has a prevalence of around 1 in 500 people in the general population. However, most people with HCM have few, if any, symptoms[1].The disease most commonly presents in the second or third decade of life but may present at any age. HCM is the most common cause of sudden unexpected death in childhood and in young athletes[2].

Treatment approaches vary depending on symptoms and risk of sudden disease. People with HCM often need to make lifestyle changes, such as limiting their activity, to adjust for their disease. European Society of Cardiology (ESC) Guidelines on hypertrophic cardiomyopathy[3] recommend that people with symptomatic disease, predominately with left ventricular outflow tract obstruction, receive beta-blockers to reduce symptoms and obstruction. If beta-blockers are ineffective or contraindicated, non-dihydropyridine calcium channel blockers (such as verapamil and diltiazem) are suitable alternatives. Disopyramide, alone or in combination with either beta-blockers or non-dihydropyridine calcium channel blockers, can also be considered. NICE technology appraisal 913 recommends the use of mavacamtem in adults who have a New York Heart Association class of 2 to 3 if it is an add-on to individually optimised standard care that includes beta-blockers, non-dihydropyridine calcium-channel blockers or disopyramide, unless these are contraindicated. If severe symptoms persist despite maximally tolerated medical therapy, people may be offered surgical myectomy or non-surgical reduction of the myocardial septum (NICE interventional procedures guidance 40). For people with obstructive HCM who progress to heart failure, the only ESC guideline recommended treatment options are those that manage left ventricular outflow obstruction. People with HCM, regardless of the presence of obstruction, should undergo clinical risk assessment for sudden cardiac death risk. In cases where individuals are considered to be at high risk of arrhythmias

and sudden cardiac death, implanted devices such as a pacemaker or an implantable cardioverter defibrillator may be used.

The technology

Aficamten (brand name unknown, Cytokinetics UK) does not currently have a marketing authorisation in the UK for treating symptomatic obstructive hypertrophic cardiomyopathy. It has been studied in clinical trials alongside established care compared with metoprolol succinate and placebo in people with symptomatic obstructive hypertrophic cardiomyopathy.

Intervention(s)	Aficamten
Population(s)	Adults with symptomatic obstructive hypertrophic cardiomyopathy
Comparators	• Mavacamten in combination with standard care • Individually optimised standard care without aficamten or mavacamten
Outcomes	The outcome measures to be considered include: • response rates • mortality • cardiovascular events • cardiovascular related mortality • exercise capacity • oxygen consumption • patient-reported symptom severity • change in NYHA class • change in left ventricular ejection fraction • adverse effects of treatment • health-related quality of life.

Economic analysis	The reference case stipulates that the cost effectiveness of treatments should be expressed in terms of incremental cost per quality-adjusted life year. If the technology is likely to provide similar or greater health benefits at similar or lower cost than technologies recommended in published NICE technology appraisal guidance for the same indication, a cost-comparison may be carried out. The reference case stipulates that the time horizon for estimating clinical and cost effectiveness should be sufficiently long to reflect any differences in costs or outcomes between the technologies being compared. Costs will be considered from an NHS and Personal Social Services perspective. The availability of any commercial arrangements for the intervention, comparator and subsequent treatment technologies will be taken into account.
Other considerations	Guidance will only be issued in accordance with the marketing authorisation. Where the wording of the therapeutic indication does not include specific treatment combinations, guidance will be issued only in the context of the evidence that has underpinned the marketing authorisation granted by the regulator.
Related NICE recommendations	**Related technology appraisals:** Mavacamten for treating symptomatic obstructive hypertrophic cardiomyopathy (2023). NICE Technology Appraisal 913. Implantable cardioverter defibrillators and cardiac resynchronisation therapy for arrhythmias and heart failure (2014). NICE Technology Appraisal 314. **Related NICE guidelines:** Chronic heart failure in adults: diagnosis and management (2018). NICE guideline 106. **Related interventional procedures:** Non-surgical reduction of the myocardial septum (2004). NICE interventional procedures guidance 40. **Related quality standards:** Chronic heart failure in adults (2011). NICE quality standard 9.
Related National Policy	NHS England 2013/14 NHS Standard contract for cardiology: Inherited cardiac conditions (All ages) The NHS Long Term Plan (2019) NHS Long Term Plan

	NHS England (2023) Manual for prescribed specialist services (2023/2024)

Questions for consultation

Will aficamten be used in the same position in the treatment pathway as mavacamten?

Will aficamten have the same restrictions on use (i.e. NYHA class) as mavacamten?

Will aficamten be used in combination with standard care?

Where do you consider aficamten will fit into the existing care pathway for symptomatic obstructive hypertrophic cardiomyopathy?

Please select from the following, will aficamten be:
A. Prescribed in primary care with routine follow-up in primary care
B. Prescribed in secondary care with routine follow-up in primary care
C. Prescribed in secondary care with routine follow-up in secondary care
D. Other (please give details):
For comparators and subsequent treatments, please detail if the setting for prescribing and routine follow-up differs from the intervention.

Would aficamten be a candidate for managed access?

Do you consider that the use of aficamten can result in any potential substantial health-related benefits that are unlikely to be included in the QALY calculation?

Please identify the nature of the data which you understand to be available to enable the committee to take account of these benefits.

NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular protected characteristics and others. Please let us know if you think that the proposed remit and scope may need changing in order to meet these aims. In particular, please tell us if the proposed remit and scope:

- could exclude from full consideration any people protected by the equality legislation who fall within the patient population for which aficamten will be licensed;
- could lead to recommendations that have a different impact on people protected by the equality legislation than on the wider population, e.g. by making it more difficult in practice for a specific group to access the technology;
- could have any adverse impact on people with a particular disability or disabilities.

Please tell us what evidence should be obtained to enable the committee to identify and consider such impacts.

NICE is considering evaluating this technology through its cost comparison evaluation process.
Please provide comments on the appropriateness of appraising this topic through this process.
(Information on NICE's health technology evaluation processes is available at https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-technology-appraisal-guidance/changes-to-health-technology-evaluation).

Technologies can be evaluated through the cost-comparison process if they are expected to provide similar or greater health benefits, at a similar or lower cost, compared with technologies that have been previously recommended (as an option) in published NICE guidance for the same indication. Companies can propose cost-comparison topics to NICE at any stage during topic selection and scoping. NICE will route technologies for evaluation through the cost-comparison process if it is agreed during scoping that the process is an appropriate route to establish the clinical and cost effectiveness of the technology.

NICE's health technology evaluations: the manual states the methods to be used where a cost comparison case is made.

- Is the technology likely to be similar in its clinical effectiveness and resource use to any of the comparators? Or in what way is it different to the comparators?
- Will the intervention be used in the same place in the treatment pathway as the comparator(s)? Have there been any major changes to the treatment pathway recently? If so, please describe.
- Will the intervention be used to treat the same population as the comparator(s)?
- Overall is the technology likely to offer similar or improved health benefits compared with the comparators?
- Would it be appropriate to use the cost-comparison methodology for this topic?

References

1. Hypertrophic cardiomyopathy. British Heart Foundation. Accessed September 2025.
2. Cardiomyopathy. NHS. Accessed September 2025.
3. Arbelo E., Protonotarios A., Gimeno J.R., et al. (2023). 2023 ESC Guidelines for the management of cardiomyopathies: Developed by the task force on the management of cardiomyopathies of the European Society of Cardiology (ESC). European Heart Journal 44(37) pg 3503-3626