NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Health Technology Evaluation

Acoramidis for treating transthyretin-related amyloidosis cardiomyopathy ID6354

Final scope

Remit/evaluation objective

To appraise the clinical and cost effectiveness of acoramidis within its marketing authorisation for treating transthyretin-related amyloidosis cardiomyopathy.

Background

Transthyretin amyloidosis (ATTR) is caused by abnormal transthyretin (TTR) proteins being produced by the liver, which accumulate as amyloid deposits (fibrils) in the tissues of the body. These amyloid fibrils can disrupt the structure and damage the function of the affected tissues.[1] ATTR can be associated with either cardiac symptoms (cardiomyopathy) or neurological symptoms (polyneuropathy) but some people can have both.[2] Transthyretin amyloidosis cardiomyopathy (ATTR-CM) is a type of transthyretin amyloidosis in which most fibrils accumulate in the heart, causing the heart tissue to thicken and stiffen.[3] There are two types of ATTR-CM:

- Wild-type ATTR-CM is the more common of the 2 types.[4] It mostly affects older individuals and is more commonly diagnosed in men than women.[5]

- Hereditary ATTR-CM (also known as familial or variant amyloid cardiomyopathy) is caused by inherited mutations in the TTR gene. The most prevalent variant in the UK is Val122Ile (15% of all TTR mutations) and less commonly Thr60Ala (2%) and Val30Met (1%).[4] People with African or Caribbean family backgrounds are more likely to have the Val122Ile variant. Among people with hereditary ATTR-CM, the poorest survival is seen in people with the Val122Ile variant.[4]

Symptoms of ATTR-CM can include shortness of breath, palpitations and abnormal heart rhythms (most frequently atrial fibrillation or atrial flutter), ankle swelling, fatigue, fainting and chest pain.

ATTR-CM is a progressive disease with symptoms usually starting after the age of 70 years in people with wildtype ATTR-CM, after the age of 60 years in people with the Val122Ile and Thr60Ala variants or after the age of 30 in people with the Val30Met variant.[6] Death in most people with ATTR-CM is from sudden death and progressive heart failure.[3] In England, around 1,500 people have been diagnosed with ATTR-CM.[7]

NICE technology appraisal guidance 984 recommends tafamidis for treating transthyretin amyloid cardiomyopathy in adults. Other current treatment options for ATTR-CM, such as diuretics, focus on symptom management and supportive care.

The technology

Acoramidis (Beyonttra, Bayer) has a marketing authorisation in the UK for treating wild-type or variant ATTR-CM.

Intervention	Acoramidis
Population	Adults with transthyretin-related amyloidosis cardiomyopathy (ATTR-CM)
Subgroups	If the evidence allows, the following subgroups will be considered: • severity of heart failure (such as by New York Heart Classification class or National Amyloidosis Centre staging) • wild type or hereditary ATTR-CM
Comparator	• tafamidis
Outcomes	The outcome measures to be considered include: • overall survival • cardiovascular-related mortality • cardiac function (such as global longitudinal strain or brain natriuretic peptide [BNP] level) • outpatient diuretic intensification • serum transthyretin and transthyretin stabilisation • cardiovascular-related hospitalisation • functional exercise capacity • signs and symptoms of heart failure (such as breathlessness) • adverse effects of treatment • health-related quality of life (of patients and carers)

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. As 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:** Tafamidis for treating transthyretin amyloidosis with cardiomyopathy (2023) NICE technology appraisal guidance 984. **Related technology appraisals in development:** Vutrisiran for treating transthyretin-related amyloidosis cardiomyopathy. NICE technology appraisal guidance [ID6470] Publication expected November 2025.

References

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3. Tsang C, Huda A, Norman M et al (2023) Detecting transthyretin amyloid cardiomyopathy (ATTR-CM) using machine learning: an evaluation of the performance of an algorithm in a UK setting. BMJ Open 1;13(10):e070028.

4. Porcari A, Razvi Y, Masi A et al (2023) Prevalence, characteristics and outcomes of older patients with hereditary versus wild-type transthyretin amyloid cardiomyopathy. European Journal of Heart Failure 25(4):515-24.

5. Gillmore JD, Damy T, Fontana M et al (2018) A new staging system for cardiac transthyretin amyloidosis. European Heart Journal 7;39(30):2799-806.

6. Patel KS, Hawkins PN (2015) Cardiac amyloidosis: where are we today? Journal of Internal Medicine. 278(2):126-44.

7. NHS England (2024) First ever life-saving treatment for rare heart condition available on the NHS. Accessed February 2025.