

Plain English Summary

Tafamidis for treating transthyretin amyloid cardiomyopathy

What does the guidance say?

Tafamidis is not recommended for government subsidy for patients with transthyretin amyloid cardiomyopathy.

Why was it not recommended for subsidy?

ACE evaluates how well a treatment works in relation to how much it costs compared to other treatments. Tafamidis was not recommended for subsidy because its benefits for patients with transthyretin amyloid cardiomyopathy do not justify its cost. If you need tafamidis for your condition, you can speak to a medical social worker to find out if there is other financial assistance available to help with the cost of treatment.

What is transthyretin amyloid cardiomyopathy?

Transthyretin amyloid cardiomyopathy (ATTR-CM) occurs when a protein called transthyretin builds up abnormally in the heart muscles. Normally, transthyretin helps to transport the thyroid hormone and vitamin A throughout the body. However, when it becomes unstable and breaks down due to gene changes (mutations) or ageing, it forms deposits, mainly in the heart.

Over time, these deposits can cause the heart muscles to become thick and stiff. This makes it harder for the heart to pump blood effectively to the rest of the body and may eventually lead to heart failure. Symptoms of ATTR-CM usually develop slowly and can include difficulty breathing, fatigue, swelling in the legs or abdomen, and an irregular heartbeat.

Abnormal transthyretin proteins can also build up in the nerves and other organs, causing other symptoms such as pain or numbness in the hands, lower back and legs.

What is tafamidis?

Tafamidis belongs to a group of medicines called transthyretin stabilisers. It attaches to the transthyretin protein and prevents deposits from building up in the body. It is taken orally.



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Is this the right treatment for me?

Your doctor may recommend you have tafamidis if other drugs cannot adequately control your condition. Your doctor should give you clear information, listen to your views and concerns, and talk to you about your treatment options.

Some of the questions you may want to ask your doctor when making decisions about your care are:

- · How will the treatment affect my day-to-day activities?
- How well does it work compared to other treatments?
- · What are the side effects and risks of treatment, and how likely are they?
- · How much does the treatment cost?
- How long will I need to be on the treatment for?
- What happens if the treatment stops working?
- What happens if I do not want to have treatment?

Published: 2 January 2024

Agency for Care Effectiveness - ACE in Agency for Care Effectiveness (ACE)

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This summary is not, and should not be regarded as, a substitute for professional or medical advice.

Please seek the advice of a qualified healthcare professional about any medical condition.

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