

# Medicine: tafamidis (brand name: Vyndaqel®)

## Resubmission

Pfizer Limited

The Scottish Medicines Consortium (SMC) has assessed tafamidis for the treatment of transthyretin amyloidosis cardiomyopathy (ATTR-CM). It is used in adults with either the hereditary or wild-type (not inherited) form of the condition. This document summarises the SMC decision and what it means for patients.

### What has SMC said?

After careful consideration, SMC has not recommended tafamidis for the treatment of ATTR-CM as described above.

SMC was unable to accept tafamidis because the evidence provided by the company was not strong enough to satisfy the committee that it offers value for money to NHSScotland. This was despite using a more [flexible approach](#)\* in the assessment, as it is for a rare condition.

### What does SMC's decision mean for patients?

Tafamidis should not normally be prescribed on the NHS in Scotland for use as described above. If a patient's healthcare professional thinks a particular patient would benefit from it, they can make a request to prescribe tafamidis. All health boards have procedures in place to consider these requests. For further information see: Medicines in Scotland: What's the right treatment for me? [www.healthcareimprovementscotland.org/medicinesbooklet.aspx](http://www.healthcareimprovementscotland.org/medicinesbooklet.aspx)



### What is tafamidis used for?

Tafamidis is used for the treatment of transthyretin amyloidosis cardiomyopathy (ATTR-CM). ATTR-CM is a rare condition in which fibres called amyloid, formed from a protein called TTR, build up in tissues around the heart. If amyloid builds up the heart doesn't work properly. There are two causes of ATTR-CM: in the hereditary form of the condition, the person has inherited a faulty copy of the TTR gene, which makes the TTR protein more likely to form amyloid; and in the wild-type condition, the TTR gene is unaffected.

\*<https://www.scottishmedicines.org.uk/how-we-decide/pace/>

## How does tafamidis work?

In patients with ATTR-CM, the TTR protein doesn't work properly and breaks up easily, with the broken pieces forming deposits called amyloid. Tafamidis helps to prevent the TTR protein from breaking down and forming amyloid deposits, thereby helping to slow the progression of the disease.

## How does SMC make its decision?

SMC carefully considers every new medicine to make sure it benefits patients and is considered to be an acceptable use of the limited resources in NHSScotland.

To do this SMC considers the following:

- Evidence from the company about how well the medicine works compared with current treatments available in Scotland, in relation to how much they will cost to buy and administer.
- Information from patient groups about the potential impact of the medicine on patients and carers.
- Advice from healthcare professionals about any benefits of the new medicine compared to current treatment, along with how the new medicine is likely to be used.

When SMC assesses a medicine it takes account of the needs of all patients in NHSScotland, not only those who may be treated with the medicine under consideration.

You can find more detailed information about the SMC assessment of tafamidis by looking at the SMC Detailed Advice Document (SMC2426).

## More information

The organisations below can provide more information and support for people with ATTR-CM and their families. SMC is not responsible for the content of any information provided by external organisations.

### UK ATTR Amyloidosis Patients' Association

 <https://ttramylodosis.uk>

### Cardiomyopathy UK

 <https://www.cardiomyopathy.org>

 0800 018 1024

You can find out more about tafamidis (Vyndaqel®) in the European public assessment report (EPAR) summary for the public by searching for the medicine name on the European Medicines Agency (EMA) website.

 <https://www.ema.europa.eu/en>