



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMADOC-1829012207-69346
EMA/H/C/002294

Vyndaqel (*tafamidis*)

A plain-language overview of Vyndaqel and why it is authorised in the EU

What is Vyndaqel and what is it used for?

Vyndaqel is a medicine used to treat transthyretin amyloidosis, a disease in which abnormal protein fibres called amyloid build up in tissues around the body.

It is used in the following patients:

- adults with early-stage nerve disease (stage 1) caused by transthyretin amyloidosis (ATTR-PN)
- adults with cardiomyopathy (ATTR-CM), where amyloid builds up in the heart. For ATTR-CM, Vyndaqel is used for both hereditary and non-hereditary types.

Senile systemic amyloidosis (a condition caused by the build-up of amyloid) is rare, and Vyndaqel was designated an 'orphan medicine' (a medicine used in rare diseases) for this condition on 8 November 2012. Further information on the orphan designation can be found on the [EMA website](#).

Vyndaqel contains the active substance tafamidis.

How is Vyndaqel used?

Treatment with Vyndaqel should be started and supervised by a doctor knowledgeable in the management of patients with the diseases Vyndaqel is used to treat. It is available as capsules and tablets to be taken by mouth once a day.

For more information about using Vyndaqel, see the package leaflet or contact your doctor or pharmacist.

How does Vyndaqel work?

In patients with transthyretin amyloidosis, a blood protein called transthyretin is defective and breaks easily. The broken protein forms a fibrous substance called amyloid that builds up in tissues and organs around the body, including around nerves and the heart, where it interferes with their normal functions.

The active substance in Vyndaqel, tafamidis, is a transthyretin stabiliser. It attaches to transthyretin, which prevents the protein from breaking up, thereby stopping the formation of amyloid and slowing down the progression of the nerve or heart disease.

Official address Domenico Scarlattilaan 6 • 1083 HS Amsterdam • The Netherlands

Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us

Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000

An agency of the European Union



What benefits of Vyndaqel have been shown in studies?

Transthyretin amyloid polyneuropathy (ATTR-PN)

Vyndaqel was compared with placebo (a dummy treatment) in 128 patients with ATTR-PN. The main measure of effectiveness was based on an assessment of the patients' nerve damage and quality of life after 18 months of treatment.

In 45% of patients taking Vyndaqel, nerve function either improved or stabilised, compared with 30% of patients taking placebo. The results did not include patients with more severe disease who left the study early because they needed to undergo a liver transplant.

Transthyretin amyloidosis with cardiomyopathy (ATTR-CM)

In a main study, Vyndaqel was shown to reduce mortality (the risk of death) due to all causes and the number of hospitalisations due to cardiovascular (heart and blood vessel) problems in patients with ATTR-CM compared with placebo.

The study involved 441 adults with wild-type or hereditary ATTR-CM who received either Vyndaqel or placebo for 30 months. The results showed that compared with placebo, treatment with Vyndaqel reduced the risk of death by around 30% and the frequency of hospitalisations due to cardiovascular problems by 32%.

Studies carried out with Vyndaqel are described in more detail in the medicine's assessment reports.

What are the side effects and restrictions with Vyndaqel?

For the full list of side effects and restrictions with Vyndaqel, see the package leaflet.

In patients with ATTR-PN, the most common side effects with Vyndaqel (which may affect more than 1 in 10 people) include urinary tract infection (infection of the structures that carry urine), diarrhoea and upper abdominal (belly) pain.

In patients with ATTR-CM, the most common side effects (which may affect up to 1 in 10 people) include diarrhoea, rash and itching.

Why is Vyndaqel authorised in the EU?

Apart from in patients with ATTR-PN who left the study because they needed a liver transplant, Vyndaqel was more effective than placebo in delaying nerve damage in patients with transthyretin amyloidosis. In patients with ATTR-CM, Vyndaqel was shown to reduce the risk of death and the number of hospitalisations due to cardiovascular problems compared with placebo. The medicine presented no major safety concerns and the risks were considered to be manageable.

The European Medicines Agency therefore decided that Vyndaqel's benefits are greater than its risks and that it can be authorised for use in the EU.

Vyndaqel has been authorised for ATTR-PN under 'exceptional circumstances'. This is because it has not been possible to obtain complete information about Vyndaqel due to the rarity of the disease. The company that makes Vyndaqel will provide any new information about the effects of Vyndaqel on disease progression and its long-term safety in certain patients every year. The Agency will review these data annually.

What measures are being taken to ensure the safe and effective use of Vyndaqel?

The company that markets Vyndaqel will provide educational material to all healthcare professionals expected to prescribe Vyndaqel. The material will include information about the need to use contraception (birth control) and avoid pregnancy while using the medicine. It also explains how pregnant women can take part in a surveillance programme and how to diagnose ATTR-CM before prescribing Vyndaqel to make sure it is the right treatment for the patient.

These materials may be made available by national competent authorities on their websites. A list of national repositories is available on the [EMA website](#).

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Vyndaqel have also been included in the summary of product characteristics and the package leaflet.

As for all medicines, data on the use of Vyndaqel are continuously monitored. Suspected side effects reported with Vyndaqel are carefully evaluated and any necessary action is taken to protect patients.

Other information about Vyndaqel

Vyndaqel received a marketing authorisation under exceptional circumstances valid throughout the EU on 16 November 2011.

Further information on Vyndaqel, including the package leaflet and assessment reports, can be found on the Agency's website: ema.europa.eu/medicines/human/EPAR/vyndaqel.

For information about the availability of this medicine in your country, contact your [national competent authority](#).

This overview was last updated in 09-2026.