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Amvuttra (*vutrisiran*)

An overview of Amvuttra and why it is authorised in the EU

What is Amvuttra and what is it used for?

Amvuttra is a medicine used to treat transthyretin amyloidosis (ATTR), a disease in which abnormal proteins called amyloids build up in tissues around the body, including around the nerves and heart.

Amvuttra is used in adults whose ATTR amyloidosis is hereditary and who have stage 1 or 2 polyneuropathy (nerve damage). Stage 1 means that the patient has weakness in the legs but is able to walk unaided. Stage 2 means that the patient can walk but needs help.

Amvuttra is also used in adults with hereditary or non-hereditary ATTR amyloidosis who have cardiomyopathy (damage to the heart muscle).

ATTR amyloidosis is rare, and Amvuttra was designated an 'orphan medicine' (a medicine used in rare diseases) on 25 May 2018. Further information on the orphan designation can be found here.

Amvuttra contains the active substance vutrisiran.

How is Amvuttra used?

Amvuttra can only be obtained with a prescription and treatment should be started and supervised by a doctor experienced in the treatment of patients with amyloidosis. Treatment should begin as early as possible after diagnosis, to avoid further progression of the disease.

The medicine is available as pre-filled syringes and is given once every 3 months by injection under the skin in the abdomen, thigh, or upper arm. Patients and caregivers may give the injection themselves after appropriate training.

Patients should take vitamin A supplements daily during treatment with Amvuttra.

For patients whose polyneuropathy progresses to stage 3 (when the patient cannot walk anymore), the doctor may continue treatment if the benefits outweigh the risks.

There are limited data available on Amvuttra in patients at severe stages of cardiomyopathy (NYHA class IV or NYHA class III plus NAC stage III). However, if patients progress to these stages while taking Amvuttra, these data suggest that they can continue treatment.

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

How does Amvuttra work?

In patients with ATTR amyloidosis, a protein called transthyretin which circulates in the blood is defective and breaks easily. The broken protein forms amyloid deposits in tissues and organs around the body, including around the nerves and heart, where it interferes with normal organ function.

The active substance in Amvuttra, vutrisiran, is a small interfering RNA (siRNA), a short piece of genetic material produced in a laboratory, which attaches to and blocks the genetic material of the cells responsible for producing transthyretin. This reduces production of defective transthyretin, thereby reducing the formation of amyloids and relieving the symptoms of ATTR amyloidosis.

What benefits of Amvuttra have been shown in studies?

In a first main study involving 164 patients with hereditary ATTR amyloidosis and stage 1 or 2 polyneuropathy Amvuttra was shown to be effective at slowing the nerve damage caused by the disease. In this study, Amvuttra was not compared with another medicine or placebo.

The main measure of effectiveness was a change in the patients' symptoms of nerve damage, as measured by a standard scale called mNIS+7, where a lower score indicates an improvement and a higher score indicates a worsening. After 18 months of treatment, the mNIS+7 score decreased by an average of around 0.5 points in patients given Amvuttra. This was compared with an average increase of 28 points in patients given placebo (a dummy treatment) in another study involving 225 patients comparing Onpattro (another hereditary ATTR amyloidosis medicine) with placebo.

The study also showed that treatment with Amvuttra was at least as effective as Onpattro at reducing transthyretin levels.

In a second main study, involving 655 patients with hereditary or non-hereditary ATTR amyloidosis and cardiomyopathy, Amvuttra was shown to be effective at reducing the risk of serious cardiovascular problems (affecting the heart and blood circulation) and death. The study looked at the number of patients who, over 3 years, died or had recurrent cardiovascular events such as hospitalisation or heart failure requiring an urgent visit. Patients given Amvuttra had an approximately 28% reduction in the risk of cardiovascular problems and death compared to patients given placebo (a dummy treatment).

What are the risks associated with Amvuttra?

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

The most common side effects with Amvuttra (which may affect up to 1 in 10 people) include reaction at the site of injection and increased blood levels of alkaline phosphatase and alanine transaminase (liver enzymes).

Why is Amvuttra authorised in the EU?

Amvuttra was shown to be effective at slowing nerve damage in patients with hereditary ATTR amyloidosis with stage 1 or stage 2 polyneuropathy. It was also shown to reduce the risks of serious cardiovascular problems and death in patients with hereditary or non-hereditary ATTR amyloidosis with cardiomyopathy. Regarding safety, the side effects are considered manageable.

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

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

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

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

Other information about Amvuttra

Amvuttra received a marketing authorisation valid throughout the EU on 15 September 2022.

Further information on Amvuttra can be found on the Agency's website: ema.europa.eu/medicines/human/EPAR/amvuttra.

This overview was last updated in 05-2025.