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Wainzua (*eplontersen*)

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

What is Wainzua and what is it used for?

Wainzua is a medicine used to treat nerve damage caused by hereditary transthyretin (ATTRv) amyloidosis, a disease in which abnormal proteins called amyloids build up in tissues around the body including around the nerves.

Wainzua is used in adults in the first two stages of the nerve damage (stage 1, when the patient is able to walk unaided, and stage 2, when the patient can still walk but needs help).

Wainzua contains the active substance eplontersen.

How is Wainzua used?

Wainzua 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 symptoms start to avoid further progression of the disease.

The medicine is given once a month as an injection under the skin in the abdomen (belly), upper thigh or upper arm, using a pre-filled pen. Patients or their carers can inject Wainzua themselves, but the first injection should be done under the guidance of a healthcare professional. Patients should take vitamin A supplements during treatment with Wainzua.

For patients whose disease progresses to stage 3 polyneuropathy, the doctor may continue treatment if the benefits outweigh the risks.

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

How does Wainzua work?

In patients with ATTRv amyloidosis, a protein called transthyretin (TTR), 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 nerves, where it interferes with their normal functions.

The active substance in Wainzua, eplontersen, is an 'antisense oligonucleotide', a short piece of synthetic genetic material that has been designed to attach to and block the genetic material of the cell

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responsible for producing transthyretin. This reduces production of transthyretin, thereby reducing the formation of amyloids and relieving the symptoms of ATTRv amyloidosis.

What benefits of Wainzua have been shown in studies?

In one main study involving 144 patients with ATTRv amyloidosis with stage 1 or 2 nerve damage, Wainzua was shown to be more effective than placebo (a dummy treatment) at slowing down the nerve damage caused by the disease.

The main measures of effectiveness were the change in patients' blood levels of TTR and changes in nerve damage and quality of life (measured using standard scales called 'mNIS+7' and 'Norfolk QoL-DN', respectively) after 65 weeks of treatment. Data from this study were compared with those for the placebo group from another study, which was conducted for Tegsedi (another ATTR amyloidosis medicine).

The main study showed that the blood level of TTR decreased by 80% in patients treated with Wainzua and by 10% in patients given placebo. The mNIS+7 score, used for assessing nerve damage, worsened by a lesser extent with Wainzua (around 3 points) than with placebo (around 26 points). Quality of life, measured by the Norfolk QoL-DN score, improved by around 6 points in patients treated with Wainzua, compared with a worsening of around 14 points in those on placebo.

What are the risks associated with Wainzua?

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

The most common side effect with Wainzua (which may affect more than 9 in 10 people) is decreased levels of vitamin A. Another common side effect with Wainzua (which may affect up to 1 in 10 people) is vomiting.

Why is Wainzua authorised in the EU?

Wainzua was shown to significantly reduce blood levels of TTR, to slow down nerve damage and to improve quality of life in patients with ATTRv amyloidosis with stage 1 or stage 2 nerve damage. Regarding safety, the side effects are usually mild to moderate in intensity and are considered manageable.

The European Medicines Agency therefore decided that Wainzua'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 Wainzua?

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

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

Other information about Wainzua

Wainzua received a marketing authorisation valid throughout the EU on 6 March 2025.

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

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