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Summary report on authorisation dated 24 October 2025

Wainzua® (active substance: eplontersen)

Authorisation in Switzerland: 14 February 2025

Solution for injection for the treatment of stage 1 or 2 polyneuropathy in adults with hereditary transthyretin amyloidosis (ATTRv).

About the medicinal product

Wainzua contains the active substance eplontersen and is administered as a solution for injection.

Wainzua is used for the treatment of adult patients with polyneuropathy[1] in the early and advanced stages (stages 1 and 2), which is caused by an inherited disorder known as hereditary transthyretin amyloidosis (ATTRv).

In this disease, mutated (defective) transthyretin (TTR)[2] is deposited in body tissues, leading to organ and nerve damage.

Since this is a rare and life-threatening disease, the medicine has been authorised as an orphan drug. The term "orphan drug" is used to refer to medicines for rare diseases.

Mode of action

The medicinal product Wainzua contains the active substance eplontersen, a so-called antisense oligonucleotide (ASO) that reduces the production of transthyretin (TTR) in the liver. As a result, less TTR enters the bloodstream, reducing the formation of harmful deposits in the tissues. This process can slow down or stop symptoms of polyneuropathy triggered by ATTRv.

[1] Polyneuropathy: a disease in which many nerves in the body are damaged, potentially resulting in tingling, numbness, muscle weakness or problems with organ function.

[2] Transthyretin (TTR): a protein that is formed mainly in the liver. It transports thyroid hormones and vitamin A in the blood.

Administration

Wainzua is a prescription-only medicine and is administered as a solution for injection in a single-use pre-filled pen[3] for subcutaneous use[4].The recommended dose is 45 mg of the active substance eplontersen (corresponding to the dose in a single-use pre-filled pen) injected once a month. The first dose should be supervised by a qualified healthcare professional in order to ensure that the patient or caregiver can perform the injection correctly. The pre-filled pen should be removed from the refrigerator at least 30 minutes before the injection to allow the active substance to reach room temperature.

Efficacy

The efficacy of Wainzua was investigated in the NEURO-TTRansform study, which was conducted at several sites. This was an open-label[5] trial based on external controls[6]. A total of 168 patients with ATTRv polyneuropathy took part in the trial. The trial participants were treated randomly in a 6:1 ratio with Wainzua or the reference product inotersen.

The efficacy of Wainzua was assessed mainly on the basis of the following criteria: the serum TTR concentration (transthyretin concentration in the blood), the improvement in the mNIS+7 score (measurement of the severity of the polyneuropathy) and the improvement in the Norfolk QoL-DN score (measurement of patients' quality of life).

The results showed that the treatment with Wainzua produced a significant improvement in all three criteria compared to the external placebo group.

Precautions, undesirable effects, & risks

Wainzua must not be used in those who are hypersensitive to the active substance or any of the excipients.

The most common undesirable effect of Wainzua (affecting more than 1 in 10 users) is a low blood level of vitamin A. Supplementation with vitamin A is therefore recommended during the treatment. Patients should also be monitored closely for symptoms of vitamin A deficiency, including visual problems.

Other common undesirable effects (affecting more than 1 in 100 users) are vomiting and reactions at the injection site, for example redness, pain and itching.

All precautions, risks, and other possible undesirable effects are listed in the Information for patients (package leaflet) and the Information for healthcare professionals.

[3] Single-use pre-filled pen: a pre-filled, ready-to-use injection device that is used once only and then discarded.

[4] Subcutaneous use: the medicinal product is injected under the skin, into the fatty tissue, using a fine needle.

[5] Open-label trial: a clinical trial without any blinding, i.e. both the trial participants and investigators (trial doctors) know which treatment is being administered.

[6] External control: comparison of the internal results with external reference data (data from other trials or registries).

Why the medicinal product has been authorised

Hereditary transthyretin-mediated amyloidosis (ATTRv) is a rare and rapidly progressive disease that can lead to serious nerve damage and premature death. The treatment options available to date have been limited.

Wainzua, with its active ingredient eplontersen, meets the need for an effective treatment for patients with ATTRv-associated polyneuropathy. Data from clinical trials have shown that Wainzua can significantly slow the progression of the disease. The most common side effects, such as low vitamin A levels, are controllable.

Taking all the risks and precautions into account, and based on the available data, the benefits of Wainzua outweigh the risks. Swissmedic has therefore authorised the medicinal product Wainzua, containing the active substance eplontersen, for use in Switzerland.

Further information on the medicinal product

Information for healthcare professionals: Information for healthcare professionals Wainzua®

Information for patients (package leaflet): Information for patients Wainzua®
Healthcare professionals can answer any further questions.

The date of revision of this text corresponds to that of the SwissPAR. New information concerning the authorised medicinal product in question will not be incorporated into the Summary report on authorisation.

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