FACTSHEET

Amyloidosis Research Consortium

Wainua™ (Eplontersen)

What is Wainua?

Wainua™ is a Ligand-Conjugated Antisense (LICA) medicine drug developed for treating patients with nerve damage (polyneuropathy) due to hereditary TTR (hATTR) amyloidosis. Wainua is designed to reduce the production of the protein transthyretin (TTR) that causes the disease.

How does Wainua work?

Wainua has been designed to reduce the production of TTR protein in the liver. It belongs to a group of drugs called Ligand-Conjugated Antisense (LICA) drugs. LICA drugs aim to prevent the production of disease-causing proteins in the body by blocking the instructions of a gene. For this reason, they are also referred to as gene silencing drugs. Genes are made up of DNA and contain the information needed to make proteins. The journey from gene to protein is complex and tightly controlled within the cell.

The target for Wainua is a molecule called RNA (ribonucleic acid), which is present in almost all living cells. The main role of RNA is to act as a messenger – it carries instructions from a gene's DNA to the cell's machinery responsible for making proteins. These instructions are then used by the cell to make a protein. Wainua attaches to and blocks RNA in liver cells, meaning the cells do not receive instructions to make more TTR protein. This reduces the amount of TTR protein being made in the liver, entering the bloodstream, and depositing in the tissues and organs of the body.

How is Wainua administered?

Wainua is a monthly self-administered subcutaneous (under the skin) injection in the stomach (abdomen), or upper thigh area. The recommended dose is 45 mg, which comes in a pre-filled autoinjector containing 0.8 mL of solution. Treatment carries on for as long as a patient continues to benefit from it. Patients will be supported to self-administer the injections at home rather than having to go into the hospital for dosing.

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What side effects might you expect?

The most commonly observed side effects in patients on the NEURO-TTRansform Phase III clinical trial – a randomized, open-label, global study to evaluate the efficacy and safety of Wainua in patients with hATTR amyloidosis with polyneuropathy - were decreased vitamin A levels, vomiting, and an increase in protein levels of the urine. Injection site reactions included erythema, pain, and itching. About 6% of participants also experienced cataract or blurred vision.

What improvements might you see with Wainua?

In the NEURO-TTRansform Phase III clinical trial, amyloidosis patients with polyneuropathy were given either Wainua or placebo (inactive drug) over the course of 40 months. Results from the trial demonstrated patients receiving Wainua:

- had significantly lowered serum transthyretin concentration
- saw statistically significant improvements in their polyneuropathy
- had statistically significant improvements in other measures looked at in the study, including overall quality of life and ability to carry out daily activities including walking

How can you or someone you care for get access to Wainua?

Wainua was approved by the FDA on December 22, 2023, for the treatment of hATTR amyloidosis polyneuropathy in the United States. Insurance coverage of Wainua will vary depending on the plan. If insurance does not provide enough coverage, there are additional programs that can provide assistance, including co-pay assistance programs by the pharmaceutical companies AstraZeneca and Ionis. More information about co-pay assistance and other programs can be found on AstraZeneca's patient support website: https://www.myaccess360.com

More information about about Wainua™ (Eplontersen) can be found at the Wainua website: https://www.wainua.com

If you have further questions, you can also visit our website at www.arci.org or contact us by phone at +1 (617) 467-5170