

AMVUTTRA™ (Vutrisiran)

What is Amvuttra?

A is an RNA interference (RNAi) drug developed for treating hereditary **transthyretin amyloidosis** (hATTR) patients **with nerve damage (polyneuropathy)**. Amvuttra is designed to reduce the production of the protein transthyretin (TTR) that causes the disease.

How does Amvuttra work?

Amvuttra is classified as a gene-silencer, which aims to inhibit production of the transthyretin (TTR) protein in the liver, thereby reducing the levels of TTR in the body, preventing amyloid build-up and organ damage.

What to expect if you or someone you care for takes Amvuttra

Amvuttra is delivered through a subcutaneous (under the skin) injection administered by a health care professional in a hospital or clinic setting once every three months. Amvuttra offers hATTR patients with polyneuropathy symptoms an additional treatment option to the two treatments currently approved: Onpattro® and Tegsedi®. It is important to discuss treatment options with your doctor to make sure that the treatment schedule and cost work for you.

What side effects might you expect?

Amvuttra was studied in HELIOS-A, a randomized, open-label, global multi-center Phase III clinical trial to **evaluate the efficacy and safety of treatment in patients with** hATTR amyloidosis with polyneuropathy. The most commonly observed side effects in patients in the HELIOS-A study included: mild short-term injection site reactions, diarrhea, pain in the extremities, and urinary tract infections. Overall, Amvuttra was well tolerated and had an encouraging safety profile.

What improvements might you see with Amvuttra?



In the HELIOS-A Phase III clinical trial, 164 adult patients with hATTR amyloidosis with polyneuropathy were given either vutrisiran via subcutaneous injection once every three months or, for comparison, patisiran (**Onpattro**) via intravenous infusion once every 3 weeks over an 18-month period. The study met all secondary endpoints at 18-months and results from the trial have shown that, by comparison, those on Amvuttra experienced:

- statistically significant improvements in neuropathy impairment, including reversal of polyneuropathy symptoms
- statistically significant improvements in other measures, including overall quality of life, nutritional status, and ability to carry out daily activities, including walking

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

Amvuttra was approved by the FDA on June 13, 2022 for the treatment of hATTR amyloidosis with polyneuropathy in the United States.

Insurance coverage of Amvuttra will vary depending on the specifics of each insurance plan. If insurance does not provide enough coverage, there are additional programs that may provide support. These include co-pay assistance programs offered by the company that makes the therapy, Alnylam Pharmaceuticals. More information about co-pay assistance and other programs can be found on Alnylam's patient support website at: www.alnylamassist.com.

More information

The HELIOS clinical trial to evaluate vutrisiran consists of two parts: HELIOS-A, described above, and HELIOS-B, a randomized, double-blind, placebo-controlled Phase III study **evaluating vutrisiran for the treatment of hereditary and wild type amyloidosis with cardiomyopathy**. Study findings from HELIOS-B are anticipated in early 2024.

For additional information, visit Alnylam's website at www.alnylam.com

We at ARC are here to help you navigate your medical journey and to support you in receiving the information and care that you need. Visit ARC's website at: www.arci.org or contact us by phone at 617-467-5170 or email at arc@arci.org.