FACTSHEET

AMVUTTRA™ (vutrisiran) for ATTR-CM

What is AMVUTTRA?

AMVUTTRA is the first gene-silencing treatment approved for patients with transthyretin amyloidosis (ATTR - both wild type and hereditary) who have heart involvement (cardiomyopathy). AMVUTTRA is a transthyretin (TTR) knockdown agent, which works with your body's natural system to stop the production of the TTR protein. AMVUTTRA was also approved in 2022 for the treatment of polyneuropathy in adults with hereditary TTR (hATTR-PN).

What is ATTR?

ATTR amyloidosis is caused by the weakening, separation and misfolding of the TTR protein, followed by a build-up in organs in the form of amyloid fibrils. When fibrils deposit in the heart, causing the heart to stiffen, this is known as ATTR cardiomyopathy, or ATTR-CM. There are two different types of ATTR amyloidosis: one is caused by an inherited mutation, called hereditary ATTR (hATTR) or variant (ATTRv), and the other is not inherited, called wildtype ATTR (ATTRwt).

How does AMVUTTRA work?

AMVUTTRA works by decreasing the amount of TTR protein made in the liver, which leads to the formation of fewer harmful amyloid fibrils depositing in the heart and causing cardiomyopathy. It acts as a gene silencer to lower the amount of TTR protein circulating in the body, and lessening disease progression.

How is AMVUTTRA administered?

AMVUTTRA is a subcutaneous (under the skin) injection administered once every three months in a hospital or clinic setting.

Clinical trial evidence for AMVUTTRA

Approval was based on the data from the HELIOS-B study. In this Phase III clinical trial, 655 adult patients with ATTR-CM (hereditary or wildtype) were given either vutrisiran or placebo via subcutaneous injection once every three months for up to 36 months (3 years). The study showed that treatment with vutrisiran substantially reduced the risk of death and cardiovascular events

relative to placebo and that patients treated with placebo saw benefits in measures of functional capacity and quality of life compared to those who received placebo.

Side effects of AMVUTTRA

For more information on side effects, you can refer to the AMVUTTRA website here.

Availability of AMVUTTRA

AMVUTTRA was approved by the FDA on March 20, 2025 for the treatment of ATTR-CM in the United States. To see if the drug is available outside the US, more info is here.

Insurance coverage of AMVUTTRA will vary depending on the particular plan. If insurance does not provide enough coverage, there are patient assistance programs that will help. This includes the Alnylam patient support program, Alnylam Assist. You can learn more about this program on Alnylam's website at alnylamassist.com or call 1-833-256-2748.

If you are on Medicare, AMVUTTRA will be covered under Medicare Part B.

More information

For more information you can visit ARC at www.arci.org. If you have further questions, call 617-467-5170 or by email at support@arci.org.