



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

21 July 2022
EMA/CHMP/637065/2022
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion¹ (initial authorisation)

Amvuttra

vutrisiran

On 21 July 2022, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Amvuttra², intended for treatment of hereditary transthyretin-mediated (hATTR) amyloidosis. Amvuttra was designated as an orphan medicinal product on 25 May 2018. The applicant for this medicinal product is Alnylam Netherlands B.V.

Amvuttra will be available as a 25 mg solution for injection subcutaneously once every 3 months. The active substance of Amvuttra is vutrisiran, a double-stranded small interfering ribonucleic acid (siRNA) that specifically targets variant and wild-type transthyretin (TTR) mRNA. The TTR gene is mutated in hATTR amyloidosis, resulting in an ubiquitous accumulation of TTR protein fragments as amyloid deposits in multiple organs. By reducing TTR production in the liver, Amvuttra reduces amyloid deposition and potentially clears existing deposits, thereby halting or even reversing disease progression.

Amvuttra has shown clinically relevant benefits for both the neurological symptoms of hATTR amyloidosis and patient quality of life. The most common side effects are pain in the extremities and arthralgia.

The full indication is: treatment of hereditary transthyretin-mediated amyloidosis (hATTR amyloidosis) in adult patients with stage 1 or stage 2 polyneuropathy.

Amvuttra should be prescribed by physicians experienced in the treatment of amyloidosis.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and made available in all official European Union languages after the marketing authorisation has been granted by the European Commission.

¹ Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion

² This product was designated as an orphan medicine during its development. EMA will now review the information available to date to determine if the orphan designation can be maintained

