26 July 2018
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Committee for Medicinal Products for Human Use (CHMP)

**Summary of opinion[1] (initial authorisation)**

# Onpattro

patisiran

On 26 July 2018, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Onpattro, intended for the treatment of hereditary transthyretin-mediated amyloidosis. Onpattro, which was designated as an orphan medicinal product on 15 April 2011, was reviewed under EMA's accelerated assessment programme. The applicant for this medicinal product is Alnylam Netherlands B.V.

Onpattro will be available as a 2 mg/mL concentrate for solution for infusion. The active substance of Onpattro is patisiran, a double-stranded small interfering ribonucleic acid (siRNA) that specifically targets all mutant and wild-type transthyretin (TTR). The TTR gene is mutated in hereditary transthyretin amyloidosis, resulting in ubiquitous accumulation of TTR protein fragments as amyloid deposits in multiple organs. Patisiran causes the catalytic degradation of TTR messenger RNA (mRNA) in the liver, resulting in significant reductions of serum TTR protein and so reducing amyloid deposition.

Onpattro has shown clinically relevant effects on both the neurological components of the disease and on quality of life, as well as a positive impact on cardiac parameters. The most common side effects are peripheral oedema and infusion-related reactions.

The full indication is: "Onpattro is indicated for the treatment of hereditary transthyretin-mediated amyloidosis (hATTR amyloidosis) in adult patients with stage 1 or stage 2 polyneuropathy". It is proposed that Onpattro be prescribed by physicians knowledgeable in the management 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

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
**Telephone** +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5520
**Send a question via our website** www.ema.europa.eu/contact