31 May 2018
EMA/CHMP/288734/2018
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Tegsedi

inotersen

On 31 May 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 Tegsedi, intended for the treatment of hereditary transthyretin amyloidosis. Tegsedi, which was designated as an orphan medicinal product on 26 March 2014, was reviewed under EMA's accelerated assessment programme. The applicant for this medicinal product is IONIS USA Ltd.

Tegsedi will be available as a 284-mg solution for injection. The active substance of Tegsedi is inotersen, an antisense oligonucleotide inhibitor of both mutant and wild-type human 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. Inotersen selectively binds to the TTR messenger RNA (mRNA) and causes its degradation. This prevents the synthesis of TTR protein in the liver, resulting in significant reductions in the levels of TTR in the circulation and so reducing amyloid deposition.

Tegsedi has shown clinically relevant effects on both the neurological components of the disease and on quality of life. The most important side effects are injection site reactions, thrombocytopenia and glomerulonephritis.

The full indication is: "Treatment of stage 1 or stage 2 polyneuropathy in adult patients with hereditary transthyretin amyloidosis (hATTR)"

It is proposed that Tegsedi be prescribed by physicians experienced in the treatment of hereditary transthyretin 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

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