



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

15 September 2016
EMA/CHMP/542534/2016
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion¹ (initial authorisation)

Chenodeoxycholic acid sigma-tau

chenodeoxycholic acid

On 15 September 2016, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation under exceptional circumstances for the medicinal product Chenodeoxycholic acid sigma-tau, intended for the treatment of cerebrotendinous xanthomatosis. Chenodeoxycholic acid sigma-tau was designated as an orphan medicinal product on 16 December 2014. The applicant for this medicinal product is Sigma-tau Arzneimittel GmbH.

Chenodeoxycholic acid sigma-tau will be available as 250 mg hard capsules. The active substance of Chenodeoxycholic acid sigma-tau is chenodeoxycholic acid, a bile acid preparation (ATC code: A05AA01) that works as replacement therapy to restore the feedback inhibition lost due to the deficiency of endogenous chenodeoxycholic acid.

The benefits with Chenodeoxycholic acid sigma-tau are its ability to reduce serum levels of cholestanol and improve symptoms of the disease. The most common side effects are hepatic adverse events.

Chenodeoxycholic acid sigma-tau is a hybrid medicine² of Xenbilox which has been authorised in the EU since 1999. Chenodeoxycholic acid sigma-tau contains the same active substance as Xenbilox, but it is authorised for a different indication.

The full indication is: "Treatment of inborn errors of primary bile acid synthesis due to sterol 27 hydroxylase deficiency (presenting as cerebrotendinous xanthomatosis) in infants, children and adolescents aged 1 month to 18 years and adults". It is proposed that Chenodeoxycholic acid sigma-tau be prescribed by physicians experienced in the management of cerebrotendinous xanthomatosis or inborn errors of primary bile acid synthesis.

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 Hybrid applications rely in part on the results of pre-clinical tests and clinical trials for a reference product and in part on new data.

