21 February 2013
EMA/CHMP/633855/2012
Committee for medicinal products for human use (CHMP)

Summary of opinion[1] (initial authorisation)

Pheburane

sodium phenylbutyrate

On 21 February 2013 the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Pheburane, 483mg/g, granules, intended for the treatment of chronic management of urea cycle disorders.

The applicant for this medicinal product is Lucane Pharma. They may request a re-examination of any CHMP opinion, provided they notify the European Medicines Agency in writing of their intention within 15 days of receipt of the opinion.

The active substance of Pheburane is sodium phenylbutyrate, it belongs to the therapeutic class of the various alimentary tract and metabolism products with ATC code A16AX03. It reduces the levels of nitrogen in the body. This is achieved after sodium phenylbutyrate is converted in the body into a substance called phenylacetate. Phenylacetate combines with the amino acid glutamine, which contains nitrogen, to form a substance that can be removed from the body by the kidneys. This allows the levels of nitrogen in the body to decrease, reducing the amount of ammonia produced.

Pheburane is a hybrid of Ammonaps which has been authorised in the EU since 8 December 1999. Studies have demonstrated the satisfactory quality of Pheburane and its bioequivalence with the reference product Ammonaps.

A pharmacovigilance plan for Pheburane will be implemented as part of the marketing authorisation.

The approved indication is: “Pheburane is indicated as adjunctive therapy in the chronic management of urea cycle disorders, involving deficiencies of carbamylphosphate synthetase, ornithine transcarbamylase or argininosuccinate synthetase. It is indicated in all patients with neonatal-onset presentation (complete enzyme deficiencies, presenting within the first 28 days of life). It is also indicated in patients with late-onset disease (partial enzyme deficiencies, presenting after the first month of life) who have a history of hyperammonaemic encephalopathy.”

[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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It is proposed that Pheburane is prescribed by physicians experienced in the treatment of urea cycle disorders.

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 will be available in all official European Union languages after the marketing authorisation has been granted by the European Commission.

The CHMP, on the basis of quality, safety and efficacy data submitted, considers there to be a favourable benefit to risk balance for Pheburane and therefore recommends the granting of the marketing authorisation.