EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

24 September 2015
EMA/628694/2015
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Ravicti

glycerol phenylbutyrate

On 24 September 2015 the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product RAVICTI, intended for the treatment of adult and paediatric patients ≥2 months of age with urea cycle disorders including deficiencies of carbamoyl phosphate-synthase-I, ornithine carbamoyltransferase, argininosuccinate synthetase, argininosuccinate lyase, arginase I and ornithine translocase deficiency hyperornithinaemia-hyperammonaemia homocitrullinuria syndrome who cannot be managed by dietary protein restriction and/or amino acid supplementation alone.

RAVICTI was designated as an orphan medicinal product on 10 June 2010. The applicant for this medicinal product is Horizon Therapeutics Limited.

RAVICTI will be available as 1.1 g/ml Oral liquid. The active substance of RAVICTI is glycerol phenylbutyrate, metabolism products (ATC code: A16AX09). Glycerol phenylbutyrate is a nitrogen-binding medicinal product.

The benefits with RAVICTI are its ability to reduce the blood ammonia levels. The most common side effects are abdominal pain, nausea, diarrhoea, and/or headache. The most frequently reported adverse reactions were diarrhoea, flatulence, and headache (8.8% each); decreased appetite (7.0%), vomiting (6.1%); and fatigue, nausea and, skin odour abnormal (5.3% each).These reactions usually disappear within a few days even if treatment is continued.

The full indication is: *" RAVICTI is indicated for use as adjunctive therapy for chronic management of adult and paediatric patients ≥2 months of age with urea cycle disorders (UCDs) including deficiencies of carbamoyl phosphate-synthase-I (CPS), ornithine carbamoyltransferase (OTC), argininosuccinate synthetase (ASS), argininosuccinate lyase (ASL), arginase I (ARG) and ornithine translocase deficiency hyperornithinaemia-hyperammonaemia homocitrullinuria syndrome (HHH) who cannot be managed by dietary protein restriction and/or amino acid supplementation alone.*

RAVICTI must be used with dietary protein restriction and, in some cases, dietary supplements (e.g., essential amino acids, arginine, citrulline, protein-free calorie supplements). "

It is proposed that RAVICTI be prescribed by physicians experienced in the management of urea cycle

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