



## Final Appraisal Recommendation

Advice No: 1819 – December 2019

### Glycerol phenylbutyrate (Ravicti®) 1.1 g/ml

#### Submission by Immedica

#### Recommendation of AWMSG

Glycerol phenylbutyrate (Ravicti®) is recommended as an option for use within NHS Wales for use as an adjunctive therapy for chronic management of patients with urea cycle disorders including deficiencies of: carbamoyl phosphate synthetase 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 (for example, essential amino acids, arginine, citrulline, protein-free calorie supplements).

This recommendation applies only in circumstances where the approved Wales Patient Access Scheme (WPAS) is utilised or where the list/contract price is equivalent or lower than the PAS/WPAS price.

#### Additional note(s):

- AWMSG considered glycerol phenylbutyrate (Ravicti®) satisfied the AWMSG criteria for ultra-orphan status.

In reaching the above recommendation AWMSG has taken account of the appraisal documentation prepared by the AWMSG Secretariat (reference number 2127), which includes the AWMSG Secretariat Assessment Report (ASAR), the Preliminary Appraisal Recommendation (PAR) and the applicant company's response to the PAR, clinical expert opinion (where available), the views of patients/patient carers (where available) and the lay member perspective.

This recommendation has been ratified by Welsh Government and will be considered for review every three years.

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