All Wales Medicines Strategy Group

Grŵp Strategaeth Meddyginiaethau Cymru Gyfan

Final Appraisal Recommendation

Advice No: 3413 – November 2013

Sodium phenylbutyrate (Pheburane®) 483 mg/g granules

Limited submission by Lucane Pharma

Recommendation of AWMSG

Sodium phenylbutyrate (Pheburane®) is recommended as an option for use within NHS Wales as adjunctive therapy in the chronic management of urea-cycle disorders, involving deficiencies of carbamylphosphate synthetase, ornithine transcarbamylase or argininosuccinate synthetase; in all patients with neonatal-onset presentation (complete enzyme deficiencies, presenting within the first 28 days of life) and in patients with late-onset disease (partial enzyme deficiencies, presenting after the first month of life) who have a history of hyperammonaemic encephalopathy.

In reaching the above recommendation AWMSG has taken account of the appraisal documentation prepared by the AWMSG Secretariat (reference number 2227), 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.

Marketing authorisation holder on first issue:	Lucane Pharma
Date of first issue	November 2013
Last reviewed	December 2016