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RESULTS FROM A NATIONWIDE COHORT TEMPORARY UTILIZATION AUTHORIZATION (ATU) SURVEY OF PATIENTS IN FRANCE TREATED WITH PHEBURANE® (SODIUM PHENYLBUTYRATE) TASTE-MASKED GRANULES

Yves Kibleur,[1] Dries Dobbelaere,[2] Nathalie Guffon,[3] Magali Barth,[4] Anais Brassier,[5] Lucane Pharma[1]. *[1 2]Reference Center for Inherited Metabolic Diseases in Child and Adulthood, University Children's Hospital Jeanne de Flandre; [3]Hôpital Femme – Mère – Enfant, Centre de Référence des maladies héréditaires du métabolisme; [4]Centre de référence maladies métaboliques - CHU Angers; [5]Ma.M.E.A – Centre de référence maladies métaboliques Hôpital Necker*

10.1136/archdischild-2015-308634.33

Objectives To describe a nationwide system for pre-marketing follow-up (cohort ATU protocol, i.e. "therapeutic utilization") of a new taste-masked formulation of sodium phenylbutyrate (NaPB) granules (Pheburane®) in France and to analyse safety and efficacy in this group of patients with urea cycle disease (UCD).

Methods In October 2012 a cohort ATU was established in France to monitor the use of Pheburane® on a named-patient basis. All UCD treated patients were included in a follow-up protocol developed by the Laboratory (Lucane Pharma) and the French Medicines Agency (ANSM) which recorded demographics, dosing characteristics of NaPB, concomitant medications, adverse events and clinical outcome. Following the granting of the EU Marketing Authorization, the cohort ATU was terminated, approximately one year after its initiation, as the product was launched on the French market.

Results Ease of administration and acceptability were much better with the new taste-masked formulation than with the previous treatment. No episode of metabolic decompensation was observed during 3 to 11 months of Pheburane® treatment and the range of ammonia and glutamine levels improved and remained within the normal range. No adverse events were reported with Pheburane®.

Conclusions The recently developed taste-masked formulation of NaPB granules improved the quality of life for UCD patients. This may translate into improved compliance, efficacy and safety, which may be demonstrated either in further studies or in the post marketing use of the product.