

SUMMARY

sodium phenylbutyrate

PHEBURANE 350 mg/ml

oral solution

Inclusion on list: First listing

Range supplement

Adopted by the Transparency Committee on 18 December 2024

Summary of opinion

Favourable opinion for reimbursement as adjunctive therapy in the chronic management of urea cycle disorders, involving deficiencies of carbamylphosphate synthetase, ornithine transcarbamylase or argininosuccinate synthetase (see the complete wording of the MA indication).

No clinical added value of the new oral solution form compared to the granule form of PHEBURANE (sodium phenylbutyrate) already available.

Transparency Committee's conclusions**Clinical Benefit**

- ➔ Urea cycle disorders are very rare metabolic diseases. In the absence of treatment, these metabolic disorders cause brain damage and may be fatal.
- ➔ PHEBURANE 350 mg/ml (sodium phenylbutyrate) oral solution is a symptomatic treatment.
- ➔ The efficacy/adverse effects ratio is high.
- ➔ There are therapeutic alternatives containing the same active substance.
- ➔ This proprietary medicinal product is a background therapy for urea cycle disorders, involving deficiencies of carbamylphosphate synthetase, ornithine transcarbamylase or argininosuccinate synthetase, in addition to a very strict low-protein diet.

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→ Public health benefit

PHEBURANE 350 mg/ml (sodium phenylbutyrate) oral solution is unlikely to have an additional impact on public health compared to PHEBURANE 483 mg/ml (sodium phenylbutyrate) granules.

The Committee considers that the clinical benefit of PHEBURANE 350 mg/ml (sodium phenylbutyrate) oral solution is substantial in the MA indication.

The Committee issues a favourable opinion for inclusion in the list of covered drugs for inpatients approved for use in the indication and at the MA dosages.

Clinical Added Value

PHEBURANE 350 mg/ml (sodium phenylbutyrate) oral solution is a range supplement that does not provide any clinical added value (CAV V) compared to the form already listed.