Health and Social Care Board

Interface Pharmacist Network Specialist Medicines

Betaine for homocystinuria

Information Sheet

Introduction

Betaine occurs naturally in the body as a metabolite and is present in small amounts in foods such as sugarbeet, spinach, cereals, and seafood. Betaine is a licensed drug (some unlicensed versions are also available) which has been used for many years on a named-patient basis as a supplement to other therapies such as vitamin B6, vitamin B12, folate and a specific diet for the treatment of classical homocystinuria in neonates, infants, children and adults. There are no licensed comparator products available.

Classical homocystinuria is a genetic disorder of metabolism of the amino acid methionine, used by the body for growth and repair. Methionine is normally broken down to the amino acid homocysteine and then to cysteine. Patients with homocystinuria are missing an enzyme that converts homocysteine into cysteine in the liver leading to very elevated plasma homocysteine levels. This imbalance can lead to mental retardation, ocular abnormalities, osteoporosis, premature atherosclerosis and thromboembolic disease. There are two different pathways of metabolism of homocysteine. To treat homocystinuria, excess homocysteine is remethylated to methionine, and Betaine acts as the methyl group donor in this process.

Dosage and Administration

Adults and Children >10 yrs old: 3g orally twice daily adjusted according to response; max 10g twice daily. Children <10 yrs old: 50mg/kg twice daily, dose and frequency adjusted according to response; max 75mg/kg twice daily.

The dosage is increased to lower total homocysteine levels and reduce free homocysteine to undetectable levels. Response usually occurs within a month.

Available as

Route	Dosage Forms	Strength	Brand Names	Supplier
Oral	Powder for oral solution	500mg/1ml when reconstituted	Unlicensed	Special Products Ltd Tel: 01932 690325
Oral	Powder for oral solution	100mg, 150mg or 1g per level scoopful provided	Cystadane®	Orphan Medical UK Tel: 01491 414 333
Oral	Tablets	500mg	Unlicensed	IDIS Ltd Tel: 01932824000

Monitoring Requirements

Monitoring is performed by the specialist at clinic appointments which are generally every 6 months. This involves:

- measurement of the plasma concentration of homocysteine (to determine the response to treatment and any dose adjustment required)
- measurement of plasma methionine levels at start of treatment and periodically thereafter (as cerebral oedema has been reported in patients with hypermethioninemia).

Adverse Effects, Precautions and Contraindications

Increased levels of methionine are commonly reported.

The following are all listed as occurring uncommonly: anorexia, agitation, depression, irritability, personality disorder, disturbed sleep, dental disorders, diarrhoea, glossitis, nausea, stomach discomfort, vomiting, hair loss, hives, abnormal skin odour, urinary incontinence.

Common Drug Interactions

No interaction studies have been performed. However, based on in vitro data, betaine might interact with amino acid mixtures and medicinal products containing vigabatrin and GABA analogues.

Communication

For any queries relating to this patient's treatment with betaine, please contact the specialist.

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