Velaglucerase alfa

- **DRUG ACTION** Velaglucerase alfa is an enzyme produced by recombinant DNA technology that is administered as enzyme replacement therapy for the treatment of type I Gaucher's disease.

- **INDICATIONS AND DOSE**

Type I Gaucher's disease (specialist use only)

▸ BY INTRAVENOUS INFUSION

- Child 4–17 years: Initially 60 units/kg every 2 weeks; adjusted according to response to 15–60 units/kg every 2 weeks

- **SIDE-EFFECTS**

▸ **Common or very common** Arthralgia · asthenia · chest discomfort · dizziness · dyspnoea · fever · flushing · gastrointestinal discomfort · headache · hypersensitivity · hypertension · hypotension · infusion related reaction · nausea · pain · skin reactions · tachycardia

SIDE-EFFECTS, FURTHER INFORMATION Infusion-related reactions are very common; manage by slowing the infusion rate, or interrupting the infusion, or minimise by pre-treatment with an antihistamine, antipyretic, or corticosteroid—consult product literature.

- **PREGNANCY** Manufacturer advises use with caution—limited information available.
- **BREAST FEEDING** Manufacturer advises use with caution—no information available.
- **MONITORING REQUIREMENTS** Monitor immunoglobulin G (IgG) antibody concentration in severe infusion-related reactions or if there is a lack or loss of effect with velaglucerase alfa.
- **DIRECTIONS FOR ADMINISTRATION** For *intravenous infusion*, reconstitute each 400-unit vial with 4.3 mL water for injections; dilute requisite dose in 100 mL Sodium Chloride 0.9% and give over 60 minutes through a 0.22 micron filter; start infusion within 24 hours of reconstitution.

- **MEDICINAL FORMS** There can be variation in the licensing of different medicines containing the same drug.

Powder for solution for infusion

ELECTROLYTES: May contain Sodium

▸ **VPRIV** (Shire Pharmaceuticals Ltd)

Velaglucerase alfa 400 unit VPRIV 400units powder for solution for infusion vials | 1 vial PoM £1,410.20

3.6 Homocystinuria

METHYL DONORS

Betaine

27-Apr-2019

- **INDICATIONS AND DOSE**

Adjunctive treatment of homocystinuria involving deficiencies or defects in cystathionine beta-synthase, 5,10-methylene-tetrahydrofolate reductase, or cobalamin cofactor metabolism (specialist use only)

▸ BY MOUTH

- Neonate: Initially 50 mg/kg twice daily (max. per dose 75 mg/kg), adjusted according to response; maximum 150 mg/kg per day.

- Child 1 month–9 years: Initially 50 mg/kg twice daily (max. per dose 75 mg/kg), adjusted according to response; maximum 150 mg/kg per day
- Child 10–17 years: 3 g twice daily (max. per dose 10 g), adjusted according to response; maximum 20 g per day

- **SIDE-EFFECTS**

▸ **Uncommon** Abdominal discomfort · agitation · alopecia · appetite decreased · brain oedema · diarrhoea · glossitis · irritability · nausea · skin reactions · urinary incontinence · vomiting

- **PREGNANCY** Manufacturer advises avoid unless essential—limited information available.
- **BREAST FEEDING** Manufacturer advises caution—no information available.
- **MONITORING REQUIREMENTS** Monitor plasma-methionine concentration before and during treatment—interrupt treatment if symptoms of cerebral oedema occur.
- **DIRECTIONS FOR ADMINISTRATION** Powder should be mixed with water, juice, milk, formula, or food until completely dissolved and taken immediately; measuring spoons are provided to measure 1 g, 150 mg, and 100 mg of *Cystadane*® powder.
- **PRESCRIBING AND DISPENSING INFORMATION** Betaine should be used in conjunction with dietary restrictions and may be given with supplements of Vitamin B_{12}, pyridoxine, and folate under specialist advice.
- **NATIONAL FUNDING/ACCESS DECISIONS**

Scottish Medicines Consortium (SMC) decisions

SMC No. 407/07

The *Scottish Medicines Consortium* has advised (August 2010) that betaine anhydrous (*Cystadane*®) is accepted for restricted use within NHS Scotland for the adjunctive treatment of homocystinuria involving deficiencies or defects in cystathionine beta-synthase, 5,10-methylene-tetrahydrofolate reductase, or cobalamin cofactor metabolism in patients who are not responsive to pyridoxine treatment.

- **MEDICINAL FORMS** There can be variation in the licensing of different medicines containing the same drug. Forms available from special-order manufacturers include: tablet, oral solution

Powder

▸ **Cystadane** (Recordati Rare Diseases UK Ltd)

Betaine 1 gram per 1 gram Cystadane oral powder | 180 gram PoM £347.00 DT = £347.00

3.7 Hypophosphatasia

ENZYMES

Asfotase alfa

04-Dec-2017

- **DRUG ACTION** Asfotase alfa is a human recombinant tissue-nonspecific alkaline phosphatase that promotes mineralisation of the skeleton.

- **INDICATIONS AND DOSE**

Paediatric-onset hypophosphatasia (initiated by a specialist)

▸ BY SUBCUTANEOUS INJECTION

- Neonate: 2 mg/kg 3 times a week, alternatively 1 mg/kg 6 times a week, dosing frequency depends on body-weight—consult product literature for further information.

- Child: 2 mg/kg 3 times a week, alternatively 1 mg/kg 6 times a week, dosing frequency depends on body-weight—consult product literature for further information

- **CAUTIONS** Hypersensitivity reactions

CAUTIONS, FURTHER INFORMATION

▸ Hypersensitivity reactions Reactions, including signs and symptoms consistent with anaphylaxis, have occurred within minutes of administration and can occur in patients