Scottish Medicines Consortium (SMC) decisions
The *Scottish Medicines Consortium* has advised (December 2017) that eliglustat (*Cerdelga*®) is accepted for use within NHS Scotland for the long-term treatment of adult patients with type 1 Gaucher disease who are CYP2D6 poor metabolisers, intermediate metabolisers or extensive metabolisers. This advice is contingent upon the continuing availability of the patient access scheme in NHS Scotland or a list price that is equivalent or lower.

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

Capsule

CAUTIONARY AND ADVISORY LABELS 10

- **Cerdelga** (Sanofi) ▼
  Eliglustat (as Eliglustat tartrate) 84.4 mg Cerdelga 84mg capsules | 56 capsule PoM £19,164.96

ENZYMES

Imiglucerase

27-Apr-2020

- **DRUG ACTION** Imiglucerase is an enzyme produced by recombinant DNA technology that is administered as enzyme replacement therapy for non-neurological manifestations of type I or type III Gaucher's disease, a familial disorder affecting principally the liver, spleen, bone marrow, and lymph nodes.

- **INDICATIONS AND DOSE**

Non-neurological manifestations of type I Gaucher's disease (specialist use only) | Non-neurological manifestations of type III Gaucher's disease (specialist use only)

- BY INTRAVENOUS INFUSION
- Adult: Initially 60 units/kg every 2 weeks; maintenance, adjusted according to response, doses as low as 15 units/kg once every 2 weeks may improve haematological parameters and organomegaly

- **SIDE-EFFECTS**
- **Common or very common** Angioedema · cough · dyspnoea · hypersensitivity · skin reactions
- **Uncommon** Abdominal cramps · arthralgia · back pain · chest discomfort · chills · cyanosis · diarrhoea · dizziness · fatigue · fever · flushing · headache · hypotension · nausea · paraesthesia · tachycardia · vomiting
- **PREGNANCY** Manufacturer advises use with caution—limited information available.
- **BREAST FEEDING** No information available.
- **MONITORING REQUIREMENTS**
- Monitor for immunoglobulin G (IgG) antibodies to imiglucerase.
- When stabilised, monitor all parameters and response to treatment at intervals of 6–12 months.
- **DIRECTIONS FOR ADMINISTRATION** For *intravenous infusion* (*Cerezyme*®), give intermittently in Sodium chloride 0.9%; initially reconstitute with water for injections (200 units in 5.1 mL, 400 units in 10.2 mL) to give 40 units/mL solution; dilute requisite dose with infusion fluid to a final volume of 100–200 mL and give initial dose at a rate not exceeding 0.5 units/kg/minute, subsequent doses to be given at a rate not exceeding 1 unit/kg/minute; administer within 3 hours after 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

- **Cerezyme** (Genzyme Therapeutics Ltd)
  Imiglucerase 400 unit Cerezyme 400unit powder for solution for infusion vials | 1 vial PoM £1,071.29 (Hospital only)

Velaglucerase alfa

23-Apr-2020

- **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
- Adult: 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* (*VPRIV*®), give intermittently *in* Sodium chloride 0.9%; reconstitute each 400-unit vial with 4.3 mL water for injections to produce a 100 units/mL solution; dilute requisite dose in 100 mL infusion fluid; 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

2.6 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
- Adult: 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.