3.9 Mucopolysaccharidosis

ENZYMES

Elosulfase alfa

04-Dec-2017

- **DRUG ACTION** Elosulfase alfa is an enzyme produced by recombinant DNA technology that provides replacement therapy in conditions caused by N-acetylgalactosamine-6-sulfatase (GALNS) deficiency.

- **INDICATIONS AND DOSE**

Mucopolysaccharidosis IVA (specialist use only)

▸ BY INTRAVENOUS INFUSION

▸ Neonate: 2 mg/kg once weekly.

▸ Child: 2 mg/kg once weekly

- **CAUTIONS** Infusion-related reactions

CAUTIONS, FURTHER INFORMATION

▸ Infusion-related reactions Infusion-related reactions can occur; manufacturer advises these may be minimised by pre-treatment with an antihistamine and antipyretic, given 30-60 minutes before treatment. If reaction is severe, stop infusion and start appropriate treatment. Caution and close monitoring is advised during re-administration following a severe reaction.

- **SIDE-EFFECTS**

▸ **Common or very common** Abdominal pain · chills · diarrhoea · dizziness · dyspnoea · fever · headache · hypersensitivity · infusion related reaction · myalgia · nausea · oropharyngeal pain · vomiting

- **PREGNANCY** Manufacturer advises avoid unless essential—limited information available.

- **BREAST FEEDING** Manufacturer advises use only if potential benefit outweighs risk—present in milk in *animal* studies.

- **DIRECTIONS FOR ADMINISTRATION** For *intravenous infusion* (*Vimizim*®), give intermittently *in* Sodium chloride 0.9%; body-weight under 25 kg, dilute requisite dose to final volume of 100 mL infusion fluid and mix gently, give over 4 hours through in-line filter (0.2 micron) initially at a rate of 3 mL/hour, then increase to a rate of 6 mL/hour after 15 minutes, then increase gradually if tolerated every 15 minutes by 6 mL/hour to max. 36 mL/hour; body-weight 25 kg or over, dilute requisite dose to final volume of 250 mL and mix gently, give over 4 hours through in-line filter (0.2 micron) initially at a rate of 6 mL/hour, then increase to a rate of 12 mL/hour after 15 minutes, then increase gradually if tolerated every 15 minutes by 12 mL/hour to max. 72 mL/hour.

- **HANDLING AND STORAGE** Manufacturer advises store in a refrigerator at 2–8°C. After dilution use immediately or, if necessary, store at 2-8°C for max. 24 hours, followed by up to 24 hours at 23–27°C.

- **PATIENT AND CARER ADVICE**

Driving and skilled tasks Manufacturer advises patients and carers should be counselled about the effects on driving and performance of skilled tasks—increased risk of dizziness.

- **NATIONAL FUNDING/ACCESS DECISIONS**

NICE decisions

▸ **Elosulfase alfa for treating mucopolysaccharidosis type IVa (December 2015)** NICE HST2

Elosulfase alfa, within its marketing authorisation, is recommended for funding for treating mucopolysaccharidosis type IVa (MPS IVa) according to the conditions in the managed access agreement for elosulfase alfa.

www.nice.org.uk/guidance/HST2

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

Solution for infusion

EXCIPIENTS: May contain Polysorbates, sorbitol

ELECTROLYTES: May contain Sodium

▸ **Vimizim** (BioMarin Europe Ltd) ▼

Elosulfase alfa 1 mg per 1 ml Vimizim 5mg/5ml concentrate for solution for infusion vials | 1 vial PoM £750.00

Galsulfase

- **DRUG ACTION** Galsulfase is a recombinant form of human N-acetylgalactosamine-4-sulfatase.

- **INDICATIONS AND DOSE**

Mucopolysaccharidosis VI (specialist use only)

▸ BY INTRAVENOUS INFUSION

▸ Child 5–17 years: 1 mg/kg once weekly

- **CAUTIONS** Acute febrile illness (consider delaying treatment) · acute respiratory illness (consider delaying treatment) · infusion-related reactions can occur · respiratory disease

- **SIDE-EFFECTS** Abdominal pain · apnoea · chest pain · chills · conjunctivitis · corneal opacity · dyspnoea · ear pain · face oedema · hypertension · increased risk of infection · infusion related reaction · malaise · nasal congestion · reflexes absent · umbilical hernia

SIDE-EFFECTS, FURTHER INFORMATION Infusion-related reactions often occur, they can be managed by slowing the infusion rate or interrupting the infusion, and can be minimised by pre-treatment with an antihistamine and an antipyretic. Recurrent infusion-related reactions may require pre-treatment with a corticosteroid — consult product literature for details.

- **PREGNANCY** Manufacturer advises avoid unless essential.

- **BREAST FEEDING** Manufacturer advises avoid—no information available.

- **DIRECTIONS FOR ADMINISTRATION** For *intravenous infusion*, dilute requisite dose with Sodium Chloride 0.9% to a final volume of 250 mL and mix gently; infuse through a 0.2 micron in-line filter; give approx. 2.5% of the total volume over 1 hour, then infuse remaining volume over next 3 hours; if body-weight under 20 kg and at risk of fluid overload, dilute requisite dose in 100 mL Sodium Chloride 0.9% and give over at least 4 hours.

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

Solution for infusion

▸ **Naglazyme** (BioMarin Europe Ltd) ▼

Galsulfase 1 mg per 1 ml Naglazyme 5mg/5ml solution for infusion vials | 1 vial PoM £982.00

Idursulfase

- **DRUG ACTION** Idursulfase is an enzyme produced by recombinant DNA technology licensed for long-term replacement therapy in mucopolysaccharidosis II (Hunter syndrome), a lysosomal storage disorder caused by deficiency of iduronate-2-sulfatase.

- **INDICATIONS AND DOSE**

Mucopolysaccharidosis II (specialist use only)

▸ BY INTRAVENOUS INFUSION

▸ Child 5–17 years: 500 micrograms/kg once weekly