

IMPORTANT SAFETY INFORMATION**SAFE PRACTICE**

Mercaptamine has been confused with mercaptopurine; care must be taken to ensure the correct drug is prescribed and dispensed.

● CAUTIONS

- When used by eye Contact lens wearers
- With oral use Dose of phosphate supplement may need to be adjusted if transferring from phosphocysteamine to mercaptamine

● SIDE-EFFECTS

- Common or very common**
 - When used by eye Dry eye · eye discomfort · eye disorders · vision blurred
 - With oral use Appetite decreased · asthenia · breath odour · diarrhoea · drowsiness · encephalopathy · fever · gastroenteritis · gastrointestinal discomfort · headache · nausea · skin reactions · vomiting
- Uncommon**
 - With oral use Compression fracture · gastrointestinal ulcer · hair colour changes · hallucination · joint hyperextension · leg pain · leucopenia · musculoskeletal disorders · nephrotic syndrome · nervousness · osteopenia · seizure
- Frequency not known**
 - With oral use Depression · intracranial pressure increased · papilloedema

- ALLERGY AND CROSS-SENSITIVITY** Contra-indicated if history of hypersensitivity to penicillamine.

● PREGNANCY

- With oral use Manufacturer advises avoid—teratogenic and toxic in animal studies.

● BREAST FEEDING

- With oral use Manufacturer advises avoid—no information available.

● MONITORING REQUIREMENTS

- With oral use Leucocyte-cystine concentration and haematological monitoring required—consult product literature.
- All patients receiving mercaptamine should be registered (contact local specialist centre for details).

● DIRECTIONS FOR ADMINISTRATION

- With oral use For children under 6 years at risk of aspiration, capsules can be opened and contents sprinkled on food (at a temperature suitable for eating); avoid adding to acidic drinks (e.g. orange juice).

● PRESCRIBING AND DISPENSING INFORMATION

Mercaptamine has a very unpleasant taste and smell, which can affect compliance.

● HANDLING AND STORAGE

- When used by eye Manufacturer advises store in a refrigerator (2–8°C)—after opening store at room temperature up to 25°C for up to 7 days; protect from light.

● PATIENT AND CARER ADVICE

Medicines for Children leaflet: Mercaptamine eye drops for ocular symptoms of cystinosis www.medicinesforchildren.org.uk/mercaptamine-eye-drops-ocular-symptoms-cystinosis

● NATIONAL FUNDING/ACCESS DECISIONS**Scottish Medicines Consortium (SMC) decisions**

- With oral use The *Scottish Medicines Consortium* has advised (November 2017) that mercaptamine (*Procsybi*[®]) is **not** recommended for use within NHS Scotland for the treatment of proven nephropathic cystinosis.

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

Gastro-resistant capsule

- Procsybi** (Chiesi Ltd)

Mercaptamine (as Mercaptamine bitartrate) 25 mg Procsybi
25mg gastro-resistant capsules | 60 capsule [PoM](#) £335.97

Mercaptamine (as Mercaptamine bitartrate) 75 mg Procsybi
75mg gastro-resistant capsules | 250 capsule [PoM](#) £4,199.65

Eye drops

EXCIPIENTS: May contain Benzalkonium chloride, disodium edetate

- ▶ **Cystaran** (Imported (United States))
- Mercaptamine (as Mercaptamine hydrochloride) 4.4 mg per 1 ml** Cystaran 0.44% eye drops | 15 ml [PoM](#) [S](#)
- ▶ **Cystadrops** (Recordati Rare Diseases UK Ltd)
- Mercaptamine (as Mercaptamine hydrochloride) 3.8 mg per 1 ml** Cystadrops 3.8mg/ml eye drops | 5 ml [PoM](#) £865.00

Capsule

CAUTIONARY AND ADVISORY LABELS 21

- ▶ **Cystagon** (Recordati Rare Diseases UK Ltd)
- Mercaptamine (as Mercaptamine bitartrate) 50 mg** Cystagon
50mg capsules | 100 capsule [PoM](#) £70.00 DT = £70.00
- Mercaptamine (as Mercaptamine bitartrate) 150 mg** Cystagon
150mg capsules | 100 capsule [PoM](#) £190.00 DT = £190.00

3.4 Fabry's disease

ENZYMES

Agalsidase alfa

24-Apr-2020

- DRUG ACTION** Agalsidase alfa, an enzyme produced by recombinant DNA technology are licensed for long-term enzyme replacement therapy in Fabry's disease (a lysosomal storage disorder caused by deficiency of alpha-galactosidase A).

● INDICATIONS AND DOSE**Fabry's disease (specialist use only)**

- ▶ BY INTRAVENOUS INFUSION
- ▶ Child 7-17 years: 200 micrograms/kg every 2 weeks

- INTERACTIONS** → Appendix 1: agalsidase alfa

● SIDE-EFFECTS

- ▶ **Common or very common** Arrhythmias · asthenia · chest discomfort · chills · cough · diarrhoea · dizziness · dyspnoea · excessive tearing · fever · flushing · gastrointestinal discomfort · headache · hoarseness · hypersomnia · hypertension · increased risk of infection · influenza like illness · joint disorders · malaise · musculoskeletal discomfort · myalgia · nausea · ototoxicity · pain · palpitations · peripheral oedema · peripheral swelling · rhinorrhoea · sensation abnormal · skin reactions · taste altered · temperature sensation altered · throat complaints · tremor · vomiting
- ▶ **Uncommon** Altered smell sensation · angoedema · hypersensitivity · sensation of pressure
- ▶ **Frequency not known** Heart failure · hyperhidrosis · hypotension · myocardial ischaemia

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

- PREGNANCY** Use with caution.

- BREAST FEEDING** Use with caution—no information available.

- DIRECTIONS FOR ADMINISTRATION** Administration for *intravenous infusion*, dilute requisite dose with 100 mL Sodium Chloride 0.9% and give over 40 minutes using an in-line filter; use within 3 hours of dilution.

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

Solution for infusion

- ▶ **Replagal** (Shire Pharmaceuticals Ltd)

Agalsidase alfa 1 mg per 1 ml Replagal 3.5mg/3.5ml solution for infusion vials | 1 vial [PoM](#) £1,049.94