PDL NEW DRUG REVIEW

Proprietary Name: Cystaran®
Common Name: cysteamine ophthalmic solution 0.44%
PDL Category: Cytokine and CAM Antagonists

Summary

Indications and Usage: Treatment of corneal cystine crystal accumulation in patients with cystinosis. This is a pregnancy category C medication. The safety and efficacy of use in the pediatric population have been established. Information regarding use with a specific age was not provided in the prescribing information and no information on recommendations regarding age was found in a review of multiple references.

Dosage Forms: Sterile topical ophthalmic solution: 6.5mg/ml of cysteamine HCl, which is equivalent to 4.4mg/ml of cysteamine (0.44%). Bottles should be stored in the freezer and removed once needed (allow about 24 hrs to completely thaw).

Recommended Dosage: Instill one drop into each eye, every waking hour. The dropper tip should not touch the eyelids or surrounding area, as to prevent contamination. Discard after one week of use, as the medication is only stable for one week after thawing. Prior to use, contact lenses should be removed and not re-inserted until 15 minutes after administration of the drops. The use in those with renal impairment has not been studied.

Common Adverse Drug Reactions: *There was no placebo data available.* The most frequently reported adverse events that occurred in ≥10% of subjects were sensitivity to light, redness, eye pain/irritation, headache, and visual field defects.

Contraindications: There are no reported contraindications listed in the prescribing information.

Manufacturer: Sigma-tau

Analysis: Cysteamine, the active ingredient of Cystaran®, is a cystine-depleting agent. It converts cystine to cysteine and cysteine-cysteamine mixed disulfides to reduce corneal cystine crystal accumulation. Three studies were performed (N=300) to assess the safety and efficacy of Cystaran®, with the primary outcome being the response rate of eyes that had a reduction of at least 1 unit in the photo-rated Corneal Cystine Crystal Score (CCCS) at some point during the study when baseline CCCS ≥1, or a lack of an increase of >1 unit in CCCS throughout the study when baseline CCCS <1. Results from study 1 suggested that in eyes with a baseline of CCCS<1 the response rate was 13%, while the response rate was 32% in those eyes with a CCCS≥1. Study 2 and 3 included those with ocular cystinosis with a baseline of CCCS≥1. The response rate was 67% in study 2 and 33% in study 3. If treatment is discontinued, corneal crystals accumulate.

Cystaran® is the only FDA approved ophthalmic medication available for this condition. It is recommended that Cystaran® remain non-preferred and require prior authorization for diagnosis confirmation.

PDL Placement:

- ☐ **Preferred**
- ☒ **Non-Preferred**
- ☐ **Preferred with Conditions**

References

[1] Cystaran [package insert]. Gaithersburg, MD: Sigma-Tau: 2012.