cystaran™

(cysteamine ophthalmic solution) 0.44%

FACT SHEET

The Ocular Effects of Cystinosis

- Cystinosis is a rare metabolic disease in which the amino acid cystine enters cells, but has no transporter out of the cells. The defect in transportation results in formation of crystals within the cells, causing early cell death.[1, 2]

- Excess cystine leads to formation of crystals that can build up and damage cells throughout the body. These crystals negatively affect many body systems, especially the kidneys and eyes.[1]

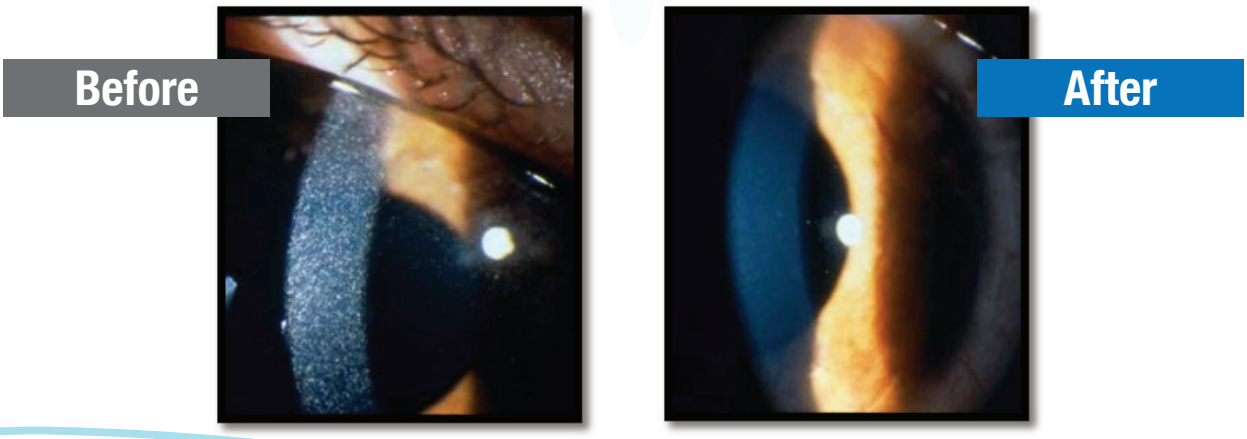
- The formation of cystine crystals within the corneas of the eyes may lead to a condition called photophobia, or sensitivity to light. Other effects include eye pain, damage to the corneas, foreign body sensation (the feeling of something in the eye), and squinting.[3, 4, 5]

- Corneal cystine crystal accumulation is a common symptom of each of the three types of cystinosis:[5, 6]
  1. Nephropathic cystinosis, which usually occurs in the first year of life, is the most common type of the disease.
  2. Intermediate cystinosis is typically diagnosed in teenagers and young adults.
  3. Nonnephropathic cystinosis typically strikes adults. This type of cystinosis is also known as ocular cystinosis and typically presents as only ocular crystals.

Treatment

- Treatment of cystinosis is focused on managing symptoms, as there is no cure.[2] An oral treatment called cysteamine has been shown to lower the amount of cystine in the cells, improve growth in children and help them develop more normally, and stabilize kidney and other functions, allowing for a more normal quality of life.[6, 7]

- However, oral cysteamine therapy is ineffective in reducing the ocular effects of cystinosis because it does not reach the corneas.[3,4]



CYSTARAN™ (cysteamine ophthalmic solution) 0.44%

- Only ophthalmic cysteamine therapy has been proven to reduce cystine crystal accumulation within the corneas. CYSTARAN™ (cysteamine ophthalmic solution) 0.44%, which is available in eye drop form, is the only FDA-approved ophthalmic treatment for corneal cystine crystal accumulation in patients with cystinosis.[8]

- The effectiveness of CYSTARAN in reducing corneal cystine crystals has been proven in numerous studies.[8]

- The most frequently reported ocular adverse reactions occurring in at least 10% of patients in CYSTARAN clinical trials were sensitivity to light, redness, eye pain/irritation, headache, and visual field defects.[8]

- Sigma-Tau Pharmaceuticals, Inc., has established the CYSTARAN Hotline, which is staffed by pharmacists, registered nurses and specialists who are trained to coordinate the delivery of CYSTARAN directly to patients, provide reimbursement support, and offer disease information. Patients, caregivers and physicians in the United States and Puerto Rico can access the CYSTARAN Hotline at 1-800-440-0473, or by visiting www.Accredo.com.

Safety:

The most frequently reported ocular adverse reactions occurring in at least 10% of patients in CYSTARAN clinical trials were sensitivity to light, redness, eye pain/irritation, headache, and visual field defects.

1 Cystinosis. Genetics Home Reference. Bethesda, MD: Lister Hill National Center for Biomedical Communications, U.S. National Library of Medicine, National Institutes of Health. http://ghr.nlm.nih.gov/condition=cystinosis. Updated April 9, 2013. Accessed April 15, 2013.

2 About cystinosis. Cystinosis Research Foundation website, 2013. http://www.natalieswish.org/About-Cystinosis. Accessed April 15, 2013.

3 Gahl WA, Kuehl EM, Iwata F, Lindblad A, Kaiser-Kupfer MI. Corneal crystals in nephropathic cystinosis: natural history and treatment with cysteamine eyedrops. Mol Genet Metab. 2000;71:100–120.

4 Kaiser-Kupfer MI, Fujikawa L, Kuwabara T, Jain S, Gahl WA. Removal of corneal crystals by topical cysteamine in nephropathic cystinosis. N Engl J Med. 1987;316:775–779.

5 Nesterova G, Gahl WA. Cystinosis. GeneReviews™, NCBI Bookshelf. National Institutes of Health web site. Available at: www.ncbi.nlm.nih.gov/books/NBK1400. Accessed April 22, 2013.

6 Cystinosis symptoms & treatment. Cystinosis Research Foundation website, 2013. http://www.cystinosis.org/symptoms-treatments. Accessed April 22, 2013.

7 National Eye Institute. Information for ophthalmologists caring for patients with cystinosis. Bethesda, MD: National Institutes of Health; April 1, 2013.

8 CYSTARAN™ (cysteamine ophthalmic solution) 0.44% prescribing information. Gaithersburg, MD: Sigma-Tau Pharmaceuticals, Inc.; 2012.