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A New Viscous Cysteamine Eye Drops Treatment for Ophthalmic Cystinosis: An Open-Label Randomized Comparative Phase III Pivotal Study.

Liang H[1], Labbé A[2], Le Mouhaër J[3], Plisson C[3], Baudouin C[2].

Author information

1 Department of Ophthalmology III, Départment Hospitalo-Universitaire View Maintain, INSERM-DHOS, and Center of Clinical Investigations 1423, Quinze-Vingts National Ophthalmology Hospital, Institut de la Vision, Université Pierre-et-Marie-Curie University Paris 06, Paris, France.

2 Department of Ophthalmology III, Départment Hospitalo-Universitaire View Maintain, INSERM-DHOS, and Center of Clinical Investigations 1423, Quinze-Vingts National Ophthalmology Hospital, Institut de la Vision, Université Pierre-et-Marie-Curie University Paris 06, Paris, France 2Department of Ophthalmology, Ambroise Paré Hospital, APHP, University of Versailles Saint-Quentin-en-Yvelines, Versailles, France.

3 Orphan Europe, Medical Affairs Department, Puteaux, France.

Abstract

PURPOSE: The purpose of this study was to evaluate the efficacy of new viscous cysteamine hydrochloride (CH) eye drops (vCH 0.55%) compared with standard CH 0.10% drops treatment.

METHODS: This was an open-label, phase III, randomized, two-arm multicenter trial conducted at two centers in France. Cystinosis patients ≥2 years old were randomized 1:1 to receive eye drops, four times per day for 90 days in both eyes. We compared the superiority in reducing corneal cystine crystal density as assessed by in vivo confocal microscopy (IVCM). We also evaluated photophobia, corneal cystine crystal scores (CCCSs), and cystine crystal depth measured by optical coherence tomography. Safety objectives were to assess adverse events (AEs), local adverse drug reactions, and ocular safety parameters.

RESULTS: We included 15 patients with vCH 0.55% and 16 patients with CH 0.10% drops for 90 days. The mean absolute change in IVCM total score at day 90 in the vCH 0.55% drops group (-4.6 ± 3.1) was significantly greater than and superior to the mean absolute change in the CH 0.10% drops group (-0.46 ± 3.38; P < 0.0001). Photophobia, CCCS, and corneal cystine crystal depth were significantly more improved in the vCH 0.55% drops group than in the CH 0.10% group. The most frequent local adverse drug reactions in both groups were stinging, burning, redness, and blurred vision.

CONCLUSIONS: vCH 0.55% was effective in reducing corneal cystine crystal density and superior to treatment with CH 0.10% drops, which offer advantages over hospital pharmacy formulations and is a more preferable and convenient treatment option.

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