

Usability Study of a new bottle for Cystaran® Eye Drops in Cystinosis Subjects currently on eyedrop therapy for Corneal Cystine Crystal Accumulation

Background: Cystinosis is a rare autosomal recessive disorder characterized by the intracellular accumulation of cystine due to a genetic mutation in the CTNS gene, resulting in defective lysosomal cystine transport. Tissue-damaging cystine crystals form within organs, including the kidney and eye. Cystine depletion by oral cysteamine is ineffective in the cornea. Clinical studies have demonstrated safety and efficacy of ophthalmic cysteamine in dissolving corneal crystals in cystinosis patients.

Study Design and Methods: An open-label study was conducted to determine the usability of a new bottle in subjects with cystinosis who were on eyedrop therapy for corneal disease. The study evaluated a bottle with a multi-dose dropper and screw cap containing cysteamine ophthalmic solution, 0.44%. 11 participants completed this study, with 3 caregivers (and the cystinosis patients they care for) and 8 cystinosis patients who self-administered the eyedrops. Virtual observation was conducted due to an ongoing pandemic. All critical tasks in the study (break the seal of the cap, open the bottle cap, administer one drop of the study drug into each eye (the prescribed dose as described in the label), close the bottle cap) were completed successfully twice by all study subjects.

Results: Ophthalmologist's observational feedback on participant performance was favorable. A minority of participants reported some difficulties opening the bottle the first time as well as squeezing the bottle to dispense a drop of the study drug, but they were able to successfully complete all the critical tasks. All participants, including those who reported difficulties, strongly agreed or agreed with the statement, "I am able to successfully place one eye drop in each eye". No participants experienced adverse events and no safety risks were identified.

Conclusions: The new bottle met all usability objectives, and was found to be acceptable by cystinosis patients and their caregivers. This study was funded by Leadiant Biosciences, Inc.