



## Cystadrops® (cysteamine) – New drug approval

- On August 19, 2020, the [FDA approved](#) Recordati Rare Diseases' [Cystadrops \(cysteamine\)](#) topical ophthalmic solution for the treatment of corneal cystine crystal deposits in adults and children with cystinosis.
- [Cystinosis](#) is a condition characterized by accumulation of the amino acid cystine within cells. Excess cystine damages cells and often forms crystals that can build up and cause problems in many organs and tissues. Cystinosis affects approximately 1 in 100,000 to 200,000 newborns worldwide.
- The efficacy and safety of Cystadrops was demonstrated in two studies: a single-arm study conducted for 5 years in 8 patients with cystinosis (OCT-1) and a randomized controlled study conducted for 90 days in 32 patients with cystinosis (CHOC). Patients received a median of 4 drops/eye/day of Cystadrops. Efficacy was assessed with In-Vivo Confocal Microscopy total score (IVCM score) by quantifying the cystine crystals in the cornea. A decrease in IVCM total score from baseline indicated a reduction in corneal crystals.
  - In the CHOC study, after 30 and 90 days of treatment with Cystadrops, 12% and 40% reduction in the total IVCM total score across all corneal layers was observed from baseline, respectively.
  - Cystadrops demonstrated greater reduction compared to the control arm at 90 days. The average reduction in IVCM total score was 4.6 in the Cystadrops arm and 0.5 in the control arm, mean difference 3.8 [95% CI: (2.1, 5.6)].
  - In the OCT-1 study, a mean decrease in corneal cystine crystal deposits of 30%, in comparison with baseline, was maintained over the 60-month period of the study.
- Warnings and precautions of Cystadrops include contamination of the dropper tip and solution, reports of benign intracranial hypertension (observed with oral cysteamine therapy), and avoidance of contact lens use.
- The most common adverse reactions ( $\geq 10\%$ ) with Cystadrops use were eye pain, blurred vision, eye irritation, ocular hyperaemia, instillation site discomfort, eye pruritus, increased lacrimation, and ocular deposits.
- The recommended dose of Cystadrops is one drop in each eye, 4 times daily during waking hours.
- Recordati Rare Diseases' launch plans for Cystadrops are pending. Cystadrops will be available as an ophthalmic solution containing 3.8 mg/mL of cysteamine (0.37%).



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