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Mol Pharm. 2016 Oct 3;13(10):3468-3477. Epub 2016 Sep 7.

### Synergistic Cysteamine Delivery Nanowafer as an Efficacious Treatment Modality for Corneal Cystinosis.

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#### Abstract

A synergy between the polymer biomaterial and drug plays an important role in enhancing the therapeutic efficacy, improving the drug stability, and minimizing the local immune responses in the development of drug delivery systems. Particularly, in the case of ocular drug delivery, the need for the development of synergistic drug delivery system becomes more pronounced because of the wet ocular mucosal surface and highly innervated cornea, which elicit a strong inflammatory response to the instilled drug formulations. This article presents the development of a synergistic cysteamine delivery nanowafer to treat corneal cystinosis. Corneal cystinosis is a rare metabolic disease that causes the accumulation of cystine crystals in the cornea resulting in corneal opacity and loss of vision. It is treated with topical cysteamine (Cys) eye drops that need to be instilled 6-12 times a day throughout the patient's life, which causes side effects such as eye pain, redness, and ocular inflammation. As a result, compliance and treatment outcomes are severely compromised. To surmount these issues, we have developed a clinically translatable Cys nanowafer (Cys-NW) that can be simply applied on the eye with a fingertip. During the course of the drug release, Cys-NW slowly dissolves and fades away. The in vivo studies in cystinosin knockout mice demonstrated twice the therapeutic efficacy of Cys-NW containing 10 µg of Cys administered once a day, compared to 44 µg of Cys as topical eye drops administered twice a day. Furthermore, Cys-NW stabilizes Cys for up to four months at room temperature compared to topical Cys eye drops that need to be frozen or refrigerated and still remain active for only 1 week. The Cys-NW, because of its enhanced therapeutic efficacy, safety profile, and extended drug stability at room temperature, can be rapidly translated to the clinic for human trials.

**KEYWORDS:** Nanowafer; corneal cystinosis; cysteamine; drug delivery

PMID: 27571217 DOI: [10.1021/acs.molpharmaceut.6b00488](https://doi.org/10.1021/acs.molpharmaceut.6b00488)

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