Cystinosis Research Foundation

Lay Abstract Template for Awardees

Please complete this lay-oriented grant abstract form which will be published on the CRF web site, in CRF Star Facts and in the CRF magazine when we announce your grant award. *Please do not exceed 400 words (no more than 1-1/4 page total).* Please submit this form electronically to nstack@cystinosisresearch.org as a Word document.

Principal Investigator (s): Xin Fan

Project Title: Liposome cysteamine carriers for eye drop formulation with long-term stability and smart release ability

Objective/Rationale: Please write a lay-oriented statement of the scientific rationale for this project. Approximately 75-85 words.

The objective of this research is to develop a novel ocular drug delivery system for cystinosis patients with corneal crystals. Early initiation and good adherence to topical cysteamine are critical for ocular cystinosis treatment. However, the strict dosing regimen and poor stability of topical cysteamine are inconvenient and add to the burden of therapy, which make adherence difficult for many patients. Here we propose a novel eye drop formulation with smart drug carriers for improving cysteamine stability and reducing dosing frequency.

Project Description: Please write a brief, lay-oriented description of how you will carry out the project. Approximately 125-135 words.

We want to provide initial proof of concept that our topical cysteamine formulation could maintain long-term cysteamine stability and provide sustained drug release with reduced dosage frequency to improve patient adherence. We will first demonstrate the inhibition effect of our formulation on the oxidation of cysteamine, this oxidation inhibition effect will contribute to the improved stability of cysteamine. Longer-term cysteamine stability in our drug delivery system will be studied. Next, in vitro cell studies and drug release studies will be used to evaluate the bioadhesive property and smart release ability of our formulation, which are key factors to maintain high drug stability during storage, but trigger release of cysteamine once applied to the eye.

Relevance to the Understanding and/or Treatment of Cystinosis: Please explain how the project will impact cystinosis treatment or increase our understanding of cystinosis. Approximately 75-80 words.

Our eye drop formulation with smart drug carriers would overcome the limitations of currently ocular cystinosis treatment with topical application of cysteamine. The main disadvantage is the extremely frequent administration requirement. In addition, cysteamine is highly susceptible to oxidative degradation to lose the therapeutic effect. Our goal is to develop an eye drop formulation of cysteamine that can maintain cysteamine stability over 30 days and reduce dosage frequency to once per day.

Anticipated Outcome: Please write a lay-oriented description of what you expect to learn/discover. Approximately 75-80 words.

We hope to develop an eye drop formulation that addresses the barriers to drug stability and patient adherence. We anticipate our eye drop formulation will maintain at least 90% stability of cysteamine for 30 days at room temperature. We also expect the eye drop will be safe, show high adhesion to mucus-producing cell, and achieve in vitro cysteamine release within the therapeutic window for 24 hours.