
PROGRESS REPORT

Project title: Molecular Mechanism of Cystinosis

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Objectives:

In this study, the goal is to understand several key aspects of how membrane proteins that have important implications in cystinosis work at the molecular level.

Executive overview of progress: Suitable for public disclosure

Membrane proteins play important roles in a large variety of physiological processes. Many membrane proteins undergo significant conformational transitions, which is essential to fulfill their function. Alteration in conformational equilibrium or transition is among the fundamental mechanisms underlying dysfunction of these proteins caused by disease-related mutations. We have applied multiple approaches to characterize the conformational dynamics of membrane proteins that have important implications in cystinosis. These studies show promise to help gain insights into the cause and potential therapy for cystinosis.