

Copy of Abstract/Summary of project

Explain the relevance to cystinosis, the problems that the fellowship addresses, and why the work is important to children and young adults with cystinosis?

Patients with cystinosis lack a functional cystinosin transport protein due to mutations in their *ctns* gene. Cell and gene-based therapies require addition of a cDNA version of the gene to allow functional cystinosin to be produced. This fellowship proposal describes state-of-the-art gene repair technology correct mutations and deletions which cause cystinosis. It will provide precisely engineered cell lines which will enhance many research groups efforts to further understand the mechanisms of by which *ctns* mutations and deletions cause cystinosis. It may also benefit cell and gene-based therapies in development for treating cystinosis.