

## **Why Patient Voices Matter: Cystinosis & Revolutionizing Gene Therapies for Rare Diseases and Beyond**

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The cystinosis community may not realize it, but it is paving the way for gene therapies for both rare and common diseases. Thanks to patients and their loved ones who speak up and demand better treatments, and due to their perseverance and courage, the first cell and gene therapy approach to cystinosis in the United States has been realized in a clinical trial. This trial also marks the first demonstration at the University of California (UC) San Diego that scientists can develop a gene therapy product in academic institutions and take it all the way to the clinic for testing in patients.

The close partnership between the Cystinosis Research Foundation (CRF) and the researchers at the UC San Diego has in large part made these important milestones possible. Now, the new Gene Therapy Initiative (GTI) seeks to apply what we have learned in cystinosis to improve current gene therapy methods and catalyze the development of next-generation gene therapies for various diseases. The generous philanthropic gift to the GTI from the Nancy and Geoffrey Stack Family Foundation will allow us to identify and fund novel gene therapy projects, as well as establish an annual symposium and seminar series at UC San Diego to educate others and encourage project collaboration. The GTI is also presenting at national scientific conferences and actively engaging with other rare disease organizations, major funding agencies and companies in the gene therapy space. The message we are sharing is simple. The cystinosis community did it, and so can others.

This is an exciting time nation-wide as there is large shift to personalized medicine and focus on rare diseases. The UC San Diego GTI invites you to attend this session not only to learn about what could be on the horizon for gene therapies, but to celebrate the accomplishments of the CRF patient advocates and the science community in treating cystinosis and other diseases in the near future.