



Natalie's Wish

Dear Friends and Family

As the holidays approach, our family has so much to be grateful for.

We look back to May of this year to our annual Natalie's Wish fundraising event and we are humbled by the love, support and generosity which all of you displayed.

When Natalie secretly scribbled her wish on a napkin before her twelfth birthday – *to have my disease go away forever* – we knew that we had to act. Natalie's wish was not a wish that any twelve-year-old should have to make, and yet her wish has become our driving force.

We could not have helped Natalie begin to realize her dream without all of you. You have provided Natalie and all those who suffer from Cystinosis the greatest gift of all – **hope. Hope for better treatments and hope for a cure. With your gift of hope, everything is possible!**

We wanted to update you on some of the exciting events that have taken place over the last few months.

Natalie's Wish Fundraiser—An Extraordinary Success

On May 20, 2004 more than 225 people attended the third annual event. Our guest speaker was Dr. William Gahl, Clinical Director of the National Genome Research Institute at the National Institutes of Health who spoke on the need for funding research for rare disorders. We watched in awe as over \$254,000 was raised during the evening's Fund-A-Cure segment. Special thanks goes to the overwhelming generosity of Michael and Lynette Hayde and the SARES•REGIS Group who matched, dollar for dollar, all amounts raised during the Fund-A-Cure. **To date, \$650,000 has been raised, which is so close to our 2004 goal of \$750,000.**

Our Scientific Review Board

The Cystinosis Research Foundation in conjunction with the Cystinosis Research Network is pleased to announce that we will be sharing the Scientific Review Board (SRB) to analyze all research proposals. The SRB is comprised of Cystinosis experts who will provide independent, objective review and funding recommendations for each research proposal submitted. We are excited about this development and will provide more details in our January 2005 newsletter.

Cystinosis Research Studies Underway

Four research studies are currently underway as a direct result of your support. A complete update will be provided in our January 2005 newsletter.

- **Dr. Ranjan Dohil** was awarded a two-year grant to study the absorption of cysteamine in the intestinal system. Data collected is necessary to understand the possibilities for a time-release medication. Currently, medications must be taken every six hours. A time-release medication would dramatically improve the quality of life for children and adults with Cystinosis.
- **Dr. Doris Trauner** was awarded a two-year grant to study myopathy (muscle wasting) in children and young adults with Cystinosis. Dr. Trauner's objective is to learn why debilitating muscle-wasting occurs and to determine a course of treatment to prevent or slow muscle deterioration.
- **Dr. Jess Thoene** – Dr. Thoene's study is focused on why lysosomal cystine causes enhanced apoptosis (early programmed cell death) in children and adults with Cystinosis.
- **Tom Jeitner, Ph.D.** – Dr. Jeitner was awarded a two-year grant to study cysteamine and its analogs to determine whether there are better methods of treatment and drug delivery for Cystinosis patients. Cysteamine is currently being studied as a treatment for Parkinson's disease and Huntington's disease.

We are so pleased with the progress being made. Two years ago none of this would have been possible. Your commitment to finding a cure has made a difference and changed the course of Cystinosis research. There is still much to be done. With your continued help we can turn Natalie's Wish into a reality. We are so thankful that you have joined us in this journey to find a cure.

We will be thinking of you this Thanksgiving as we count our blessings and remember all of you who have given so much to make Natalie's dream and the dream of all children with Cystinosis come true.

Cystinosis is a rare, inherited, metabolic disease that is characterized by the abnormal accumulation of the amino acid cystine in each cell. The build-up of cystine in the cells slowly and eventually destroys all major organs of the body including the kidneys, liver, eyes, muscles, bone marrow, thyroid and brain.

Save the Date
for next year's event:
Thursday, June 2, 2005
Four Seasons Hotel
Newport Beach