

ELEVEN YEARS OF



Miracles & Milestones

2013 NATALIE'S WISH CELEBRATION

Dear Friends,

It was just eleven years ago that Natalie scribbled her birthday wish on a napkin – *to have my disease go away forever.*

Following that heartbreaking moment we established the Cystinosis Research Foundation with the sole purpose of finding better treatments and ultimately a cure for cystinosis. We never anticipated how successful the foundation would be in such a short time. With the help of friends like you, CRF has raised more than \$20,000,000 since 2003. Every dollar continues to go directly to research.

We have made remarkable research advances and have achieved some exceptional milestones, but we have more work to do. With each passing day, cystinosis continues to cause damage to Natalie's body. Cystinosis causes the amino acid cystine to abnormally accumulate in the cell. As it does, it crystalizes the cell leading to premature cell death. The end result is the slow deterioration of organs in the body including the kidney, eyes, liver, thyroid gland and brain. Time is running out for our children.

This year's *Natalie's Wish Celebration* is more crucial than ever. We are on the cusp of unprecedented medical advances that hold the potential to be truly life-changing and life-saving for people with cystinosis. Our team of brilliant doctors and researchers are close to historic discoveries. We have asked for your support in the past and we hope you will join us again for our *Miracles and Milestones* celebration.

As parents of a child with cystinosis, we live in a constant state of worry and oftentimes, with a sense of desperation about Natalie's future. What gives us and other parents of children with cystinosis hope, is the research advances we have made and the real possibility that CRF-funded researchers will find a cure.

With your love and generosity, we know we will make Natalie's wish a reality for every person with cystinosis.

We sincerely hope you will join us on Saturday, April 20 for what promises to be our biggest celebration ever.

With a grateful heart, *Nancy and Jeff Stack*

Images on cover: Natalie Stack, l-r, age 11 months, 11 years, 21 years





NANCY AND JEFF STACK & ALEX AND NATALIE

INVITE YOU TO JOIN THEM FOR

Miracles & Milestones

2013 NATALIE'S WISH CELEBRATION

Honoring the children and adults who are affected by cystinosis and the cystinosis research community for its commitment to our children.

Featuring a special performance by *American Idol* winner Taylor Hicks and the All-American Boys Chorus.

Saturday, April 20, 2013

6 pm Cocktail Reception

7:15 pm Program and Dinner

Cocktail Attire

Balboa Bay Club and Resort

1221 West Coast Highway

Newport Beach, CA 92663



Currently, there is no cure for cystinosis, *but there is hope.*

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

Medication is available to control some of the symptoms of this insidious disease, but cystinosis remains incurable.

Cystinosis afflicts approximately 500 people, mostly children, in North America and fewer than 2,000 worldwide. It is one of the 7,000 rare or "orphan" diseases in the United States that collectively affects almost 30 million Americans.

Federal funding for research on cystinosis and other rare diseases is virtually non-existent and most pharmaceutical companies remain uninterested because financial rewards are too small.

Yet, while there is only a small number of patients who suffer from any given "orphan" disease, knowledge gained by studying one disease often leads to advancements in other rare diseases and more prevalent and well-known disorders.

Cysteamine, currently the medicine used to treat cystinosis patients, is also in clinical trials as a possible treatment for Huntington's disease, Parkinson's disease and NASH (fatty liver disease), which affect millions of people worldwide.



CYSTINOSIS RESEARCH FOUNDATION

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The Cystinosis Research Foundation is a non-profit, tax-exempt entity pursuant to Section 501(c)3. Federal Tax ID #32-0067668
100% of the funds raised support cystinosis research. Your gift is tax deductible.

The Natalie's Wish invitation package was completely underwritten by friends of the Cystinosis Research Foundation.