Natalie's Wish

Star light, star bright,
First star I see tonight
I wish I may, I wish I might
Have the wish, I wish tonight.

FOR FRIENDS OF NATALIE STACK AND SUPPORTERS OF THE CYSTINOSIS RESEARCH FOUNDATION ★ FEBRUARY 2006

From Natalie

- *I started 9th grade in September. I recently started playing basketball for my high school JV team. So far, it is my favorite part of high school!*

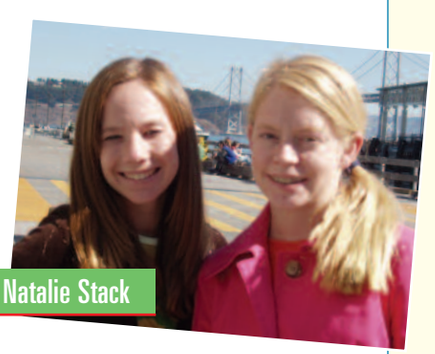
Last year, I started a new medication for my eyes. In October I had an appointment at the National Institutes of Health (NIH) in Bethesda, Maryland. I met with Dr. Tsilou because my eyes always felt itchy and sometimes it felt like I had an eyelash in my eyes even though I didn't. Dr. Tsilou is the doctor who examines the eyes of kids with Cystinosis. I had an early morning appointment at the NIH. They examined my eyes, dilated them and took pictures of the crystals covering my corneas.

The doctor saw lots and lots of cystine crystals in my eyes. That was not a good thing because if more crystals form, I could lose my eye sight or damage my corneas. That would be awful and it would make it hard to live a normal life. When I found out that I could go blind if I did not take the eye drops, it scared me and I knew I had to use the eye drops every day.

The doctors told me that I had to take the eye drops 8–12 times every day or else the cystine crystals would keep forming in my eyes. If I don't take them regularly the crystals won't go away. If I take the drops all the time, the crystals will eventually dissolve but the hassle is that I have to take them every day forever. I am glad there are eye drops and I have a treatment. I have learned to take the eyedrops by myself.

Thank you everyone for making such a difference and helping all of us with Cystinosis have hope for a better future.

Love
Natalie Stack



Alex and Natalie Stack

Dear Friends and Family

In February 2003, on Natalie's twelfth birthday, she shared her secret wish with me: **"To have my disease go away forever."** Because of all of you, that wish is one step closer to becoming a reality. Our fourth annual Natalie's Wish event on June 2, 2005 was an extraordinary success.

This amazing evening began with best-selling author and journalist, Morton Kondracke sharing the story of his late wife Milly's courageous battle with Parkinson's disease. Mr. Kondracke emphasized the need for funding medical research especially for orphan diseases like Cystinosis.

After Mr. Kondracke's presentation, we watched in amazement as our first live auction raised $98,000 for Cystinosis research. Following the auction, the highlight of the evening was the Fund-A-Cure segment where we again were astonished as more than $290,000 was donated for research. By the end of the night, over $825,000 was raised for Cystinosis research. **100 percent of every dollar donated to the Cystinosis Research Foundation goes to Cystinosis research**. All costs of the event and day-to-day costs of the foundation are completely underwritten. In 2005 alone more than $1,146,000 was raised for research!

> We could not do this without all of you. Your outpouring of love and support has been invaluable.

We cannot thank you enough for your support in our efforts to find better treatments and ultimately a cure for Cystinosis. Your support has allowed us to fund a number of multi-year research studies, something that had been impossible in the past due to lack of long-term funding. Currently there are six research studies underway and one study being reviewed and pending approval by the Scientific Review Board. We look forward to making an announcement soon!

continued on page two

BOMA Golf Tournament for Cystinosis Research Foundation

For the second year in a row, the Building Owners and Managers Association (BOMA) of Orange County chose the Cystinosis Research Foundation as the beneficiary of their Annual Charity Golf Tournament in August, 2005. The tournament sold out with 144 participants. The live and silent auction raised over $9,000 for the Foundation.

We are grateful to our friends at BOMA-OC. Special thanks to the committee who worked so tirelessly: Bob Brans of Structural Materials, John Czorniak of Pacific Building Care, Diana Dean of Environomics West, Tom Devlin of Allied-Barton Security Service, Dale Fedele of Ampower, Seth Foster of HSG/Birdbusters, Lynn Infurchia of Belfor USA, Jeff Koscher of Advanced Restoration, Lisa Miller of CarrAmerica Realty Corp., Monique Mora of the Gale Company, Betty Pickett of Universal Protection, Mike Raring, AAA Electrical & Communications, Jon Schneider of Specialty Uniform Services, Eric Sorensen of Able Engineering, Brett Wells of Frazee Paint and Robin Jochims of BOMA-OC. Thank you!

BOMA
Building Owners and Managers Association of ORANGE COUNTY

The 2006 BOMA-OC Charity Golf Classic will be held August 2, at the Talega Golf Club in San Clemente. For details visit www.natalieswish.org/events.

I am inspired by my good pal Natalie...

David Martin Tri-ing to Find a Cure for Natalie

All of us have ideas about certain limitations and restrictions and what we can and cannot do. Often we hear "shouldn't" and "couldn't" and use those terms for not trying at all. I am inspired by my good pal Natalie who rejects those negatives and says "Yes I can." So in spite of the "shouldn't" and "can't"...I "could" and "did" complete the Lavaman Triathlon in Hawaii. Again spectators asked "who is Natalie?," "What is Cystinosis?" I was so proud to share Natalie's wish.

Thank you Natalie for allowing me to share your story and represent the Cystinosis Research Foundation.

Dear Friends and Family *continued from front page*

The Cystinosis Research Foundation (CRF) is the largest nonprofit funder of Cystinosis research. As of 2003, the CRF has funded more than $1.5 million supporting seven Cystinosis Research projects. This year more than $895,000 is available for Cystinosis research projects. We will soon announce to the medical and scientific communities that we are accepting grant proposals. On June 1, during our Natalie's Wish event, we will announce which research studies will be funded in 2006.

We are continually amazed at how blessed and fortunate we are to have such a wonderful community of family and friends. We could not do this without all of you. Your outpouring of love and support has been invaluable and we treasure every letter, email and note we receive. Your prayers have made a difference – we are moving into the next clinical phase of the slow-release medication study. Research is slow but we are continually learning new and important information about Cystinosis. It is a time of hope!

Although Natalie looks healthy on the outside, inside she is not. In October we visited the NIH because she was experiencing pain and discomfort in her eyes. The exam revealed that Natalie's corneas were covered with cystine crystals. Accumulation of the crystals causes photophobia and pain similar to the feeling of having sand in your eyes. Although the news was not good, it was not unexpected given the inevitable progression of Cystinosis. Fortunately, there are experimental eye drops available as a treatment. We are now in our fourth month of eye drops. To be effective, the drops need to be refrigerated and must be taken every waking hour a minimum of eight times a day. The drops dissolve the crystals but they must be taken for the rest of her life or the crystals will reaccumulate. Although the eye drops sting and smell, Natalie has learned to take them herself. We are grateful there is a treatment and that she is able to tolerate it.

It is not just our family that is so thankful. We receive letters from other families who have children affected by Cystinosis. So many of their children are very ill and suffering every day. They thank us for the new and continuing research funded by the CRF. It is because of all of you – our community of friends and family that have made a difference. So, on behalf of all of the families with Cystinosis, we are eternally grateful for your continued support as we move closer to improving and prolonging the lives of our children.

Your financial support has provided Natalie and others with Cystinosis the hope they so desperately need. We thank you for your commitment to our cause. We are also grateful to the many volunteers who made our Natalie's Wish event such a success. We are especially thankful for the tireless efforts of Zoe Solsby and Marylyn Milburn.

We hope that you will join us for this year's *Natalie's Wish* event on Thursday, June 1, 2006 at the Balboa Bay Club in Newport Beach. The evening promises to be inspirational and uplifting with Kevin Sharp, an award-winning vocalist and speaker, sharing *The Power of A Wish* – his story of hope and survival. We look forward to seeing you there.

Nancy and Jeff Stack

Feature on Sigma-Tau

"What's the difference between a disease affecting 300 and 300,000,000? To us, nothing.

At Sigma-Tau Pharmaceuticals, Inc., our mission is to help ensure patients with rare diseases are not left untreated. We are dedicated to providing new therapies and new hope for patients with rare diseases."

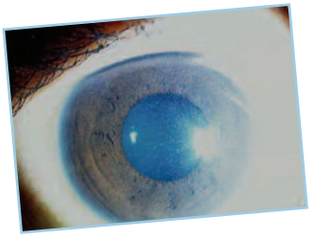
GREGG LAPOINTE, CHIEF OPERATING OFFICER, SIGMA-TAU PHARMACEUTICALS, INC.

Laura's a Picky Eater

Marybeth knew that there was something wrong with her daughter Laura almost from birth. She was a picky eater with cravings for salty and spicy food, and she was so tiny for her age that her measurements didn't make it on to the growth charts. But it wasn't until her second birthday – after being admitted to the hospital for severe dehydration following the flu – that doctors finally consulted Dr. Frederick Kaskel, a pediatric kidney specialist.

As soon as he heard Laura's story, Dr. Kaskel suspected Cystinosis. Laura's parents weren't aware at the time, but he had recently attended a lecture by Dr. William Gahl of the National Institutes of Health, who had described Laura's problem. After a battery of tests, Dr. Kaskel confirmed Laura's diagnosis of Cystinosis, and her parents were on the phone talking to Dr. Gahl, who had dedicated his life's work to this rare disease.

After the conversation, Laura's parents knew they were in capable hands. Marybeth recalls, "Instead of saying, 'Don't worry about her, she'll probably outgrow this', he asked questions and listened to my answers." She learned that Cystinosis is an inherited metabolic disease that results in an abnormal accumulation of the amino acid cystine in organs of the body. This can cause many serious complications. An oral treatment called Cystagon® can help manage the crystal accumulation in all the organs except the eye.



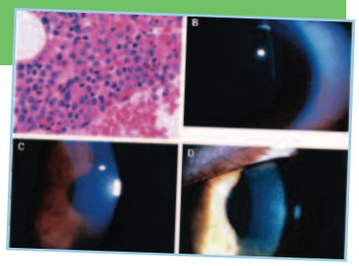
Tiny crystals of cystine begin to develop in the cornea and cause hypersensitivity to light that Laura experienced. The tiny specks shown in the bar of light are corneal cystine crystals – the result of cystine build-up.

Not long after meeting Laura and hearing her story, Dr. Gahl asked Marybeth to enroll her in an eye drop protocol study of children with Cystinosis being conducted at the NIH. Since then, and for most of her life, Laura has been involved in testing the experimental eye drops.

The eye drops are being developed by NIH with support from Sigma-Tau Pharmaceuticals, Inc. Sigma-Tau is dedicated to researching and developing treatments to improve the lives of patients and families living with rare diseases. *Simply because it's the right thing to do.*

Laura does not want to draw attention to her condition, thus the challenge of getting a busy young adult to administer the eye drops on a regular basis. The road has not been easy. Every four months for much of her life, Laura returned to the NIH for monitoring and medication adjustments.

Now 20, Laura is a normal, young adult. Early on, her family developed a philosophy for living with the disease: "We can't change what you have," her parents told her, "we have to learn to live with it."



Laura's success with her treatments was due to the dedication of her parents, clinical researchers and companies like Sigma-Tau, who are dedicated to research for treatments for rare diseases like Cystinosis. Laura's participation in the clinical study, helps other children with Cystinosis be assured that there is hope.

Life Is Looking Up for Jack

Can you imagine watching a three-year-old child guzzle an entire 20-ounce bottle of water in one gulp? Or watching your son vomit up everything he eats? His parents, Christy and Dave can.

When he was born, Jack was a big, happy baby who ate, cried, slept and played much like his older sister. As he grew, however, instead of walking like other kids, Jack continued to scoot across the floor on his bottom. His parents also noticed that he drank a lot and went through a lot of diapers. But he was a big baby, so his parents just assumed it was because of his size.

When Jack stopped eating and started vomiting regularly his parents knew that something was wrong. Visits to their pediatrician led to consultations with gastroenterologists, geneticists, nephrologists and ophthalmologists. As a result, his parents, Christy and Dave, got a crash course in obscure diseases.

Since that diagnosis at one-year-old, perspective became the battle cry of their daily lives with Cystinosis, and the word 'normal' has taken on new meaning. Jack now has a gastric feeding tube in his stomach through which he receives liquids, nutrition and medicine. He's also taking a number of medicines to manage his disease. Jack's everyday, six-hour medical routine has enabled him to grow surprisingly well, and allowed him to become one of the healthiest of all kids with Cystinosis.

But despite the lifelong medication regimens and challenges of preparing for an independent adulthood, patients like Jack can be confident that others will continue researching this disease to add to the knowledge and understanding of how to better manage it, while offering hope to others.

Dedicating resources to the NIH research is just one way that Sigma-Tau demonstrates its commitment to rare diseases. Sigma-Tau has supported patients with these diseases since 1985, when it became one of the first companies in the world to obtain an Orphan Drug approval in the United States. To Sigma-Tau, there is no greater accomplishment than helping people with rare diseases. Its mission is to help ensure that patients with rare diseases are not left untreated.

Sigma-Tau Pharmaceuticals, Inc., is one of those rare companies dedicated to discovering new therapies for patients with rare diseases.

Birthday Wishes

Fourteenth Birthday Party Benefits Cystinosis Research Foundation

Kelsey Valley, Shelby Searles, Jenny Madden and Caroline Kelter, four Harbor Day School 8th graders, who have been Natalie's classmates for nine years, decided to celebrate their fourteenth birthdays with a party to benefit the Cystinosis Research Foundation.

On Friday, May 27, 2005, Memorial Day weekend, the girls boarded the TIKI boat out of the Balboa Fun Zone with 45 classmates and seven brave Moms. They toured the harbor for five hours while dancing to the sounds of a DJ from Los Angeles who played their favorite songs.

In lieu of gifts, the girls requested that guests write a check to support *Natalie's Wish* and by the end of the evening they had raised $6,415! The girls were so thrilled they thought about making this an annual event to help Natalie's wish come true.

Kirsten's Eighth Birthday

For her eighth birthday party, Kirsten Hansen wanted to do something special. She decided to have a theatre party, with a *Grease* theme. A professional actress/director lead Kirsten and her guests in a rehearsal and then dressed them in 50's era costumes. At the end of the party the girls performed a short version of the musical *Grease* for their parents and families. However, this was not the special part. Instead of bringing presents to the party, Kirsten and her guests each made a contribution to the Cystinosis Research Foundation.

Natalie was in Kirsten's Harbor Day School family and Kirsten wanted to do something special for her during their 8th grade year. More than $1,000 was raised and the girls all had a wonderful time.

Ava's First Birthday

By Renee Carter – Ava's Mother

On April 2, 2005 our daughter Ava turned one year old. A milestone – a time to reflect on the wonderful progress in her young life – and for us, a time to honor our dear friend and a true inspiration, Natalie Stack. When our son, Nathan, had his first birthday in August of 2003 we requested "no gifts." We also asked that if guests felt compelled to give something, they make a donation on his behalf to the Cystinosis Research Foundation.

We continue to believe the best gift we can give our children is the willingness to share their blessings of good health and good fortune with those who are less fortunate. For this reason, we chose to jointly celebrate the life our youngest child, Ava, and the life of Natalie and others with Cystinosis. We are honored to include Natalie and the entire Stack family among our most celebrated of friendships.

Ava's party was wonderful. The In-n-Out Burger truck served our guests; a princess castle jumper provided a fun outlet for the kids; and a face painter and balloon twister topped off the day. A day that we'll always remember!

6 Questions for

Lynette and Michael K. Hayde

1 How did you first learn about the Cystinosis Research Foundation?

Mike and Lynette knew for many years that Natalie Stack had a rare disease. But they never approached Jeff or Nancy about it because they did not want to intrude or cause them any more pain than they were already living with. They did, however, respond to an invitation to attend a ***Natalie's Wish*** event where they first learned about Natalie's disease. It was a tough evening for them and for everyone who attended. Mike and the gentleman he sat with "were in tears" much of the evening.

2 You know the Stacks personally and professionally. Is that why you first became donors? Are there other reasons you continue to be so committed?

Mike has known Jeff for more than 20 years. They are friendly yet fierce competitors in the apartment business. But they have a great deal of respect for each other. Their business relationship is almost certainly what got Mike and Lynette to attend that first event. "We became donors because we were deeply touched as we listened that night," Lynette said. While Mike had an initial gift in mind, Lynette immediately rejected it and said, "We have to do more." Lynette is a compassionate person who believes in miracles. She also believes, "that if the mind can conceive it, then it can become a reality. As long as we are able, we will work to make Natalie's wish come true."

3 With your extensive expertise in the nonprofit world, what distinguishes the Cystinosis Research Foundation from other nonprofits? Are there other organizations that have a fundraising approach similar to CRF and its commitment to good stewardship?

Mike and Lynette decided long ago they would spend most of their time and money helping children because "they are the future." "We give to many important causes, but most of the time we don't personally know those who benefit from our giving. The Cystinosis Research Foundation presented us with a unique opportunity. Of course we understand the benefits for those who are affected by this terrible disease. But in this case we personally know someone who suffers from Cystinosis. Natalie is very special to everyone who knows her. This makes this quest extremely personal."

4 Cystinosis is a rare disease affecting only a few hundred children in the United States. How big a role does that play in your commitment to the organization?

"If not all of us, then who will take on this challenge?" asks Lynette. It is clear that the major pharmaceutical companies cannot or will not do research on a disease if the market is so small it will not generate a profit. While most of us don't fully understand or even agree with this reasoning it seems that "it's up to us to make a start. We hope the Cystinosis research we're helping to fund will provide crossover benefits for other diseases. Then the market may be large enough to entice a major pharmaceutical company into the fight."

5 As advocates for *Natalie's Wish,* what would you say to others to encourage them to be involved?

"We all have our favorite causes," but Mike and Lynette still ask their friends and colleagues to help Natalie. "This courageous young lady knows her life expectancy is short but she is still filled with joy," Lynette says. Mike and Lynette are convinced they can make a difference, and they encourage others to help as well. "If someone can look at Natalie and other children with Cystinosis and turn away, there is nothing more we can say to convince them to help," she adds.

6 You have made a large financial contribution to the CRF for Cystinosis research, what are you thoughts?

"Some would say that there's not much chance of the research being successful, but we like siding with the underdog," Mike says. An American president once got school children to collect dimes to find a cure for polio. It was a dreadful disease and seemed unconquerable. But in his youth Mike was part of that effort. Today, when the vaccination is given there is no polio. Now Mike boldly adds, "We will support Cystinosis research until we find the cure."

Friends and Families

DANIELLE HARRISON – AGE 3

Marana, Arizona

By Kathy Harrison – Danielle's Mother

Danielle was diagnosed with Cystinosis when she was 15 months old. She had been sick since birth but became very sick when she was nine months old. We took her to the doctor but he did not know what was wrong with her. She ended up in the hospital. The doctors told us that Danielle was like a "ship with a hole in it" – she would be in and out of the hospital until they could diagnose her. Fortunately, a home nurse came to visit us. She took one look at Danielle and told us to immediately take her to Phoenix Hospital before she dies. We met Dr. Haws there and he diagnosed her with Cystinosis.

When Danielle was diagnosed a year and a half ago, she had advanced rickets, high blood pressure and was diagnosed with a heart problem. Today, Danielle is 3 years old and still has the high blood pressure, a heart problem and she is beginning to have problems with her eyes. Danielle takes 13 medications and supplements every day. Her medicine schedule, which is administered seven times each day, begins at 5 am and ends at 11 pm. She weighs only 33 pounds and is 35 inches tall. She is not on the growth chart for height at all and just made the chart for weight.

Danielle has had nine hospital visits to date and has been in the hospital for renal failure several times and twice because she stopped eating. She has a G-tube so that we can more easily administer medications and nutrition. She has been at "death's door" three different times but each time she made it through.

When Danielle was first diagnosed, we did not know how rare Cystinosis was. We just wanted Danielle to be well and out of the hospital. When we learned more about Cystinosis we were devastated. As parents, the hardest part is the lack of sleep and the unknown. Now, we take things one day at time and enjoy every day with her. Our wish for Danielle is for her to have a happy, healthy, long life. Thank you for all you are doing to help find a cure for Cystinosis.

HEATHER'S STORY

Alberta, Canada

By Louise Wegerif–Heather's Mom

Sarah, Bruce (Dad), Rachel, Heather and Louise (Mom)

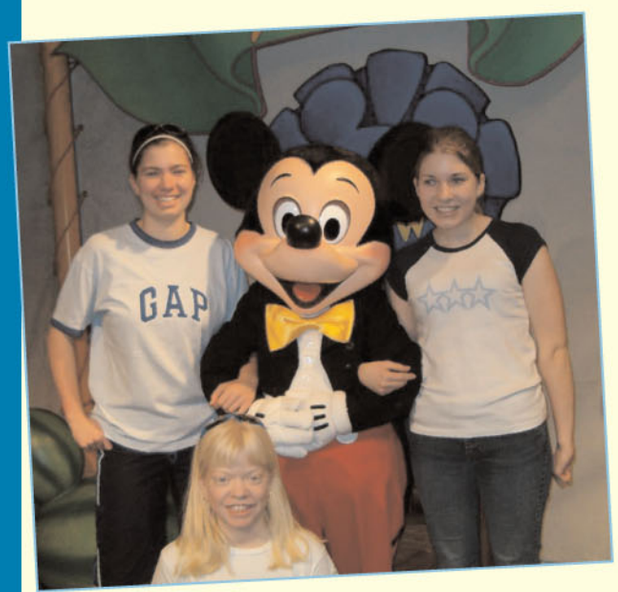
Heather is almost 15 years old and has Cystinosis. She is in the 9th grade and lives in Calgary, Alberta, Canada. She has two sisters, Sarah, age 20, and Rachel, 17, who love her dearly and would do anything for her. Sarah is quite determined to be tested to see if she is able to donate a kidney to Heather when the time comes.

Heather was diagnosed at 13 months of age, which is early. She started taking medicine to treat Cystinosis as soon as she was diagnosed. It was a big adjustment for our whole family. Currently Heather takes six medicines four times per day, two medicines twice per day, and three medicines once a day. These are either in pill form or taken through her G-tube. In addition, she also has two medicines which are given by injection once a day. On top of all that, she also is supposed to give herself the eye drops eight times per day. We are trying to teach her to be responsible for her own care, and she usually does a great job! But she often just "forgets" it's time to do her meds. Heather is so proud of her latest accomplishment: She has just started giving her injections to herself!

On the whole Heather is doing really well physically. Her kidney function is about 40 percent and she is 5'2" thanks to growth hormone, which she has been on since she was in 2nd grade. Heather loves to draw, cook, baby-sit and be silly with her sisters.

> We are so thankful for having a little angel in our midst!

As her parents, we find that one of the most difficult things is the unending and constant need to watch the clock to give her medicines. It means there isn't much room for doing anything spontaneously! The effects Cystinosis has on Heather's brain are the most upsetting to us. She struggles with reading and writing, and school is very difficult for her. We also have a difficult time with accepting those things that she will never get to do – such as going to camp, sleeping over at a friend's house, or being independent – which many would consider normal parts of growing up.



Sarah, Mickey, Rachel and Heather (below)

Our life has not been the same since the day we first heard of Cystinosis. A lot of what Cystinosis is and does is really upsetting, but our family is far the better for having Heather in it, just as she is!

We have learned so much from Heather during the past 15 years – things we probably would not have learned any other way. We are so thankful for having a little angel in our midst!

By SHANNON PAJU

Anaheim, California

2005 did not turn out like I thought it would. The transplanted kidney I received in 2002, died after only three years. I spent my nineteenth birthday in the hospital on life support.

Since June of 2005, I have been unable to eat or drink anything by mouth – and I love to cook and eat! I am "fed" and receive medications through a G-J tube. I was in the hospital more than I was at home in 2005, and I'm on hemodialysis three days a week.

Unfortunately, I am not on a list for a new kidney at this time. I hope to have surgery to repair my esophagus and after that I will be able to eat and drink by mouth again. When I recover from the surgery, I will be put back on the kidney transplant list so I can live a normal life.

When I am home, I enjoy reading cook books, spending time with my friends and playing with my puppy Nevach.

I did have good news in 2005, I had two poems published. One is printed below.

Seconds

Seconds to minutes, minutes to hours,
hours to days, days to weeks, weeks to months,
months to years...

Pain to tears, tears to fears the fears of being alone,
fears of dying alone, the fears of love,
the fears of being in love...the fear of not knowing
what these seconds minutes hours days weeks
months and years bring to you, to your life.

Friendships? Good? Bad? Relationships?
Good? Bad? Children? Healthy? Sick?
You don't know, no one knows, all you know
is the fear you feel...the fear of the unknown

The seconds, minutes, hours, days, weeks,
months and years pass and the pain grows stronger
rapist murders and terrorists run the streets and you,
not knowing where or when they will strike next.
Your fear grows so strong you cant even leave
your home and once again the fear of the
unknown sounds your mind and your soul

Seconds to minutes, minutes to hours, hours to days,
days to weeks, weeks to months, months to years,
will we ever be able to live a life not in fear?

Shannon M. Paju

Emily and Teresa Morris, Natalie's Cousins Raise Money for Cystinosis

Emily and Teresa Morris, Natalie's cousins, decided to hold their own lemonade sale fundraiser in June 2005. The girls live in Minden, Nevada and with the help of the community came together to help support *Natalie's Wish.* The sisters set up a beautiful stand to sell lemonade during the month of June at The Dance Workshop, owned by Ann and Craig Peters. The Albertson's store in South Carson donated the lemonade and cookies. Costco Wholesale provided bakery goods, and Wal-Mart donated the paper products. Linda Johnson, an Avon representative provided a beautiful gift basket for the girls to auction. Troy and Shelley Alfonso donated ice and storage for all the supplies.

The girls also created and sold inspirational bracelets. The bracelets read, *hope*, *dream*, *strength*, *faith*, *courage* and *love*. Emily and Teresa know these words are the words everyone touched by Cystinosis thinks and feels each day.

Special thanks to the entire community of Minden for supporting Emily and Teresa in their effort to help Natalie and all those with Cystinosis.

2005 Donor List

$50,000 AND MORE

Lynette and Mike Hayde
SARES•REGIS Group
Nancy and Jeff Stack

$20,000 AND MORE

Sheri and Chris Dialynas
Equity Residential Foundation
David and Suzu Neithercut
Marcy and Gerry Spector
Ginni and Kent Valley

$10,000 AND MORE

Gerson and Barbara Bakar
Diane and Bruce Bearer
Renee and Daryl Carter
Judy and David Christensen
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$5,000 AND MORE

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Don and Sonja Grant
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Susan and Mark Hillgren
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Chrissy and Rick Nicholas
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Gary Ryness
Janet Keller and Bernie Schneider
Christine and Bill Thormahlen
The Ryland Group
Sheila and Ted Weschler

Donor List

$2,500 AND MORE

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Dianne and Richard Azar
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Louise and Peter Donovan
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Linda and Kris Elftmann
J.P. Morgan Chase
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Doug and Donna Neff
Eric and Shirley Pepys
RL and David Peters
Bob and Betty Stine
Allan and Cindy Stokke
Mark Tennison
Jill and Larry Tucker
Watson Land Company
Wells Fargo Bank

$1,000 AND MORE

Bette and Wylie Aitken
Bill and Annette Albert
Ginger and Tony Allen
The Alter Family
David Ash
Bank of America
Bemus Landscape, Inc.
Jill and Blake Bertea
Lesley and Alan Beyer
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George Bystrom
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Chapman, Glucksman & Dean
Paula and Jeff Cole
Budge Collins
Corpus Christi Catholic
Christian Community
Con Am Management Corp.
Anne Marie and Bob Couglan
Ron Cox
Diane and Gene Crain
Kim and Dick Crawford
Doug and Cindy Crocker

Hugh, age 4, diagnosed prenatally

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For more information about Cystinosis, visit www.natalieswish.org.

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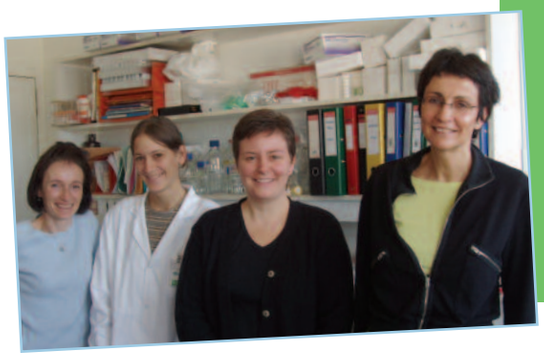

Cystinosis Research Foundation
Research Updates from the Doctors

Corinne Antignac, MD, PhD

Hospital Necker-Enfants Maades, Paris, France

Our laboratory is located at Necker Hospital in Paris, where there is a long history in the Department of Pediatric Nephrology of treating children with Cystinosis. We started working on Cystinosis in 1995, when the chromosomal localization of the gene on chromosome 17 had just been found by the International Cystinosis Consortium. We identified the gene underlying Cystinosis in 1998, in collaboration with Dr. W. van't Hoff and Dr. M. Town in London. We subsequently showed that cystinosin, as they named the Cystinosis gene product, was indeed a specific transporter of cystine located at the membrane of the lysosomes. We also characterized mutations in the Cystinosis gene in almost 150 children from all over the world. This allowed us to correlate the severity of the disease with the type of mutations and identify key regions in cystinosin. Since no animal model of Cystinosis existed, we generated a knock-out mouse model, in which the mouse gene has been inactivated. These mice present elevated cystine levels, even at birth, which increase with age. Furthermore, cystine crystals were present in various organs from the age of six months. These mice do not develop any obvious renal phenotype. In contrast, from 6–8 months of age, they show decreased activity and osteoporosis, as well as ocular lesions similar to those in patients, making them a good model to study the mechanisms of cystine accumulation and to test new treatments to decrease the cellular concentration of cystine.

Our current research projects are aimed at understanding the exact role of cystinosin in the cells and why its alteration leads to the development of Cystinosis. We are using both our animal model and cellular models we have recently developed in the lab.

The severity of the disease in the mouse varies depending on the strain of the mouse. Thus, we have transferred the mutation of the Cystinosis gene onto two pure mouse strains and are currently analyzing the mice for kidney, behavioral, bone and ocular changes. In addition, in collaboration with Dr. Minnie Sarwal at Stanford University, we are comparing the gene expression patterns between normal and Cystinosis mouse tissues to identify key cellular pathways either altered in Cystinosis or, on the contrary, involved in the protection of the cells. This could explain the lesser severity of the disease in the mouse model, and then be exploited in the long term to develop new therapeutic strategies.

Another very exciting field of our research, currently funded by the Cystinosis Research Foundation, is the study of the role of cystinosin at the cellular level. We have several reasons that make us think that cystinosin might also have a role in cystine transport in the lysosome. We are currently trying to demonstrate it using cell lines we have generated stably expressing cystinosin (normal or mutated) tagged with a fluorescent marker, which renders it easily detectable under a fluorescence microscope.

Ranjan Dohil, MD

Associate Clinical Professor
Division of Pediatric Gastroenterology and Hepatology, UCSD School of Medicine

Pharmacokinetic evaluation of cysteamine bitartrate (Cystagon™) and cystamine

When Cystagon™ was approved in 1994, the FDA did not require information about the absorption, metabolism and distribution of Cystagon™ (cysteamine bitartrate). While it was initially seen as a benefit that the FDA did not require this information before approving Cystagon™, this lack of knowledge is now limiting the development of an improved way to administer the drug. The aim of the animal study we have designed is to evaluate how much of the drug is absorbed into the blood stream and once absorbed, how much is metabolized by the liver as soon as it has been absorbed.

In addition to testing Cystagon™, we will also evaluate cystamine. Cystamine is an odorless compound, which consist of two cysteamine molecules and the results of the study will help us assess if cystamine could be used as a pro-drug. The advantage of a pro-drug may be a reduction of gastrointestinal side effects as well as the avoidance of the unpleasant body odor Cystagon™ causes. Cystamine is potentially a therapeutic option for the future. In the meantime, however, we are focused on creating a new method of delivering Cystagon™.

Controlled-release Cysteamine Therapy.

Results from our initial study would suggest that a controlled-release cysteamine therapy would be possible. We are excited about the next phase of our study, i.e., creating the actual controlled-release therapy and testing patients with Cystinosis. We recently received a commitment from Mylan Laboratories, Inc., the current provider of Cystagon™, to provide the medication necessary for this study. We appreciate their participation in helping to move this study forward.

CRF is pleased to report that the results of Dr. Dohil's absorption study will be published in the *Journal of Pediatrics* within the next few months. Once published it will be posted on our web site at www.natalieswish.org.

Progress Report: Mitchondrial Dysfunction in Cystinosis Myopathy

Doris A. Trauner, MD

Professor, Departments of Neurosciences and Pediatrics; Chief, Pediatric Neurology UCSD School of Medicine

Aims of the study:

The specific aims are to determine whether the myopathy associated with nephropathic Cystinosis is the result of mitochondrial dysfunction with resultant deficiency in respiratory transport chain function in adolescents and adults with Cystinosis, and to determine if a treatment regimen that provides additional co-factors for these enzymes will improve the strength and prevent deterioration in muscle strength.

Progress to date:

❶ Enrollment

Eleven patients (age range 16–43 years) have been enrolled in the study. One was unable to continue because of prior participation in a study of cardiac function. Of the remaining ten, one has completed the 12-month visit, eight have completed the six-month visits, and one has completed the first visit. All patients have been randomized to treatment with a "mitochondrial cocktail" or placebo. The code has not yet been broken to determine which patients have received which regimen.

❷ Results of study to date

A. Symptoms of myopathy: Only three of 11 patients complained of weakness and/or chewing and swallowing difficulties. No patient under the age of 25 had a history of weakness or easy fatigue.

B. Muscle biopsy: 11 muscle biopsies have been performed. Six had abnormal findings on light microscopy. These included fiber size variation, myofiber atrophy, ring fibers, endomysial fibrosis, vacuolar inclusions, increase in red granular staining with trichrome, increase in lipid droplets with oil red O, and type II fiber atrophy. One additional muscle biopsy was not diagnosed as abnormal, but there were findings suggestive of mild type II fiber atrophy and increase in size of lipid droplets. Electron microscopy of the muscle was available for seven patients. Only two were read as normal. Abnormal findings included filamentous bodies, ring fibers, occasional mitochondrial degeneration, and tubular intracytoplasmic inclusions. These abnormalities were present even in patients who had no complaints of muscle weakness, and who had good strength on muscle testing. Thus, myopathic changes in the muscle can be found even in asymptomatic patients with Cystinosis.

C. EMG/NCV: We have received reports of six EMG and NCV studies to date. Two patients had normal studies (20 and 21.5 years old). Three showed myopathic changes, with decreased amplitude and duration of motor unit action potentials, and numerous polyphasic potentials. Two patients had neurogenic changes, one in addition to myopathic changes and one without myopathic changes.

D. Metabolic studies: Two-thirds of the patients had low or borderline low plasma Co-enzyme Q 10 levels. The same percentage had elevated blood CPK levels, suggestive of muscle damage. All had normal muscle carnitine levels. These findings add to the pathological data and suggest that muscle may be involved in Cystinosis even without evidence of clinical symptoms, and the low Co-enzyme Q 10 levels suggest that the muscle problem may be the result of mitochondrial dysfunction.

E. Exercise testing: Results of exercise testing on all of the patients have not been analyzed yet, because the blinding in terms of placebo vs. mitochondrial "cocktail" has not been broken. We will conduct those analyses at the completion of the 12-month studies.

Future plans

❶ Complete 6- and 12-month testing.

❷ Enter new patients as they are identified. We would like a total of 20 patients.

❸ Analyze data and write manuscripts with results of study.

A Study of the Cognitive Domain of Executive Functioning in Individuals with Cystinosis

Amy M. Spilkin, PhD

Principal Investigator

Angela O. Ballantyne, PhD

Co-Investigator

Progress to date: Over the first six months we obtained Institutional Review Board approval for the study, ordered supplies, and trained a research assistant on the measures to be administered. In addition, we have successfully recruited, inducted, and tested 14 individuals with Cystinosis and 2 control participants. Hence, we have made substantial progress toward the goals of this study.

Results: On all 16 participants to date, we have collected D-KEFS, Wechsler IQs, and the age-appropriate executive functioning questionnaires (BRIEF or FrSBe). We have also scored, reliability checked, and data entered all of the test and questionnaire data collected so far. Since we are in the first quarter of this study, we have not yet analyzed any of the data.

Future plans: In the next six months, we plan to continue to recruit, induct and test individuals with Cystinosis and matched control participants, as we work towards the projected participants (i.e., 30 with Cystinosis, 30 controls.)

Because of your generosity to the Cystinosis Research Foundation we have funded and committed nearly **$1,600,000** in Cystinosis research. This makes the CRF the largest nonprofit funder of Cystinosis research in the world.

Corrine Antignac, MD, PhD
Hospital Necker-Enfants Maades, Paris, France
2005 - $78,000

Ranjan Dohil, MD
Associate Clinical Professor Division of Pediatric Gastroenterology and Hepatology, School of Medicine University of California, San Diego,
2002–2005 - $338,542
2005 - $200,000

Thomas Jeitner, PhD
Research Assistant Professor, Department of Biochemistry, Medical College of Wisconsin
2004–2005 - $92,680

Dzung H. Nguyen, PhD
Assistant Project Scientist Ajit Varki Laboratory, School of Medicine, Glycobiology Research and Training Program, University of California, San Diego
2005 - $127,000

Amy Spilkin, PhD
Assistant Project Neuroscientist Pediatric Neurology Research Group University of California, San Diego
2005 - $167,865

Jess G. Thoene, MD
Director, Hayward Genetics Center, Karen Gore Professor Pediatrics, Tulane University, School of Medicine
2002 - $100,000
2003 - $105,000
2004 - $75,000
2005 - $200,000

Doris A. Trauner, MD
Professor, Department of Neurosciences, University of California, San Diego
2004 - $97,300

As research updates become available they will be posted at www.natalieswish.org

$772,986
in Research Funded through 2005 Natalie's Wish Event

Corinne Antignac, MD, PhD
Hospital Necker-Enfants Maades, Paris, France
"Characterizations of Cystinosin Intracellular Trafficking"
$78,000 – 1-year study

Ranjan Dohil, MD
University of California, San Diego,
"Clinical Studies of Cystinosis: Attempts to Improve Treatment"
$200,000 – 2-year study

Dzung H. Nguyen, PhD
University of California, San Diego
"Development of a Rapid Method of Neutrophil/Monocyte Isolation of the Diagnosis and Monitoring of Cystinosis"
$127,121 – 2-year study

Jess G. Thoene, MD
(On hold due to hurricane Katrina, research lab destroyed)
Tulane University, School of Medicine
"Lysosomal Cystine Enhanced Apoptosis in Cultured Human Mesenchymal Stem Cells"
$200,000 – 2-year study

Amy Spilkin, PhD
University of California, San Diego
"A Study of the Cognitive Domain of Executive Functioning in Individuals with Cystinosis"
$167,865 – 2-year study

The Power of a Wish

We are excited to announce that Kevin Sharp will be the guest speaker at our Fifth Annual *Natalie's Wish* Fundraiser on Thursday, June 1, 2006 at the Balboa Bay Club in Newport Beach, California.

Kevin Sharp, award-winning vocalist, entertainer and cancer survivor, will inspire us all as he shares his experiences and the lessons he learned while battling cancer. The foundations of his strong beliefs are never giving in and never giving up. Kevin knows the most powerful gift we have in this life is – **HOPE**.

As a teenager, Kevin was a gifted athlete who excelled in several sports. He began experiencing fatigue and unexplained pain in his leg, which was later diagnosed as a rare form of bone cancer (Ewings Sarcoma) that had spread to his lungs.

As a senior in high school, Kevin was told that his chance of survival was slim. Uncertain if he would live six months, Kevin was introduced to the Make A Wish Foundation, which grants wishes to children with life-threatening illnesses. They honored his wish of meeting producer/performer David Foster, whose friendship sustained Kevin through two grueling years of chemotherapy, experimental drugs and radiation treatments. It also opened a door for Kevin to pursue his goal to become a successful country artist.

To everyone's surprise, Kevin went into remission in 1991. He has never forgotten the generosity of the Make A Wish Foundation, and currently acts as a national spokesperson for the group. He was also honored with the prestigious *Wishgranter of the Year Award*, which singled him out for the devotion, dedication and inspiration he provides the children. To date, Kevin is the only "wish child" to become the wish request of other wish kids, making his wish experience come full circle.

A platinum debut album, *Measure Of A Man;* scores of award nominations; and a string of chart-topping hits including *She's Sure Taking It Well, If You Love Somebody,* and the highly acclaimed *Nobody Knows* – which held the top spot on Billboard's Country Singles Chart for an astonishing four weeks – mark his debut as the best by a new male country singer in five years.

Kevin is a living example of the ***power of a wish*** and we are blessed to have him share his music and his story with us at ***Natalie's Wish*** on June 1, 2006.

I wish I may, I wish I might, have the wish I wish tonight.

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THURSDAY, JUNE 1, 2006

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