



KirbyGram

June 2005

The latest news on Kirby Wilson and friends and the search for a cure for Sanfilippo Syndrome

What is Sanfilippo Syndrome?

Sanfilippo Syndrome is one of seven Mucopolysaccharide (MPS) disorders. There are four different enzyme deficiencies that cause Sanfilippo. The Sanfilippo disorders are described as type A, B, C, or D. There is very little difference between the four types, though there have been a few very mild cases of the B form reported where the children have remained relatively healthy into early adult life.

Children with Sanfilippo are missing an essential enzyme that breaks down a complex body sugar called heparan sulfate. This sugar slowly builds in the brain, stopping normal development and causing hyperactivity, sleep disorders, loss of speech, dementia and typically, death before adulthood. There is no cure yet.

While Sanfilippo occurs once in 24,000 births, successful research into the disease could apply directly to many of 5,000 other genetic disorders.

KIRBY UPDATE

April 15th was a warm, bright, sunny day and thankfully, we are not just talking about the weather! It was Kirby's 14th birthday.

After a celebration at school with classmates and wearing her birthday crown, Kirby came home to her loving family to continue the party. First on the agenda was some outdoor play on her teeter-totter and swing, which always brings a smile and laughter to her face. Of course, Kirby's birthday would never be complete without her ever-favorite pizza, Coke and cake.



Sue comments, "Looking for a special gift for Kirby this year, I realized how Kirby continues to be our teacher. Brad and I are very fortunate to be able to give Kirby toys and such that interest her, but as time goes on, it becomes harder and harder to find something different and new. After finding only a few sand toys to replace the ones the dog "played with" last year, I could think of nothing else.

"Finally, it dawned on me. She not only doesn't need anything else but truly doesn't want anything else. Kirby is happiest when she is with us....playing, singing, hugging and yes, eating. She wants for nothing more. Isn't that true for us all? Are we not happiest when we are with people we love and we feel loved? So, once again, Kirby, without words, was able to enlighten our lives, brighten our spirits and warm our hearts. We are blessed by her wisdom."

FUNDRAISING NEWS

A "Sweetheart" of a Night

The Tenth Annual Sweetheart Dinner Dance was held at The Drake Hotel in Chicago on February 5th. This year, 207 "sweethearts" attended and raised more than \$61,000 for research. The Drake Hotel again honored the event with its own "Red Day," where employees wore red to signify their support of the Foundation and its mission. The "sweethearts" were even successful in convincing Michael Lerich and his talented group of musicians, who have

entertained this devoted group of supporters every year, to do an encore at the end of the evening.

The Wilsons are grateful for the steadfast support of The Drake and Michael Lerich, who for ten years have helped to ensure the success of the event, and the commitment of so many who have attended throughout the years to show their compassion for Kirby and faith in a mission Brad and Sue hold very close to their hearts.

FUNDRAISING NEWS

The Foundation Gives Thanks...

To **The LaGrange Highlands Woman's Club**, which has proven the strength of women on a mission. The Foundation was chosen as this past year's "Charity of the Year" for the second time. The club set an all-time record for fundraising and was able to present a check to Sue for \$7,000! We thank this tireless group for not only the support and confidence in the Foundation, but also its compassion for Kirby and all the other children like her. What a powerful combination!

To **Starbuck's** and **Loni Green of On-The-Spot Massage Therapy** for joining efforts and raising funds and awareness with chair massages at this popular Western Springs coffee shop. What a great blend for Kirby!

To second-graders **Meghan Golden, Sydney Stancik and Nicholas Gron** for their driveway bake sale "to raise funds for Kirby's cure." Too sweet!

To **White Hen Pantry, Kirschbaum's Bakery and Casey's Market**, all from Kirby's hometown of Western Springs, for their continuous fundraising efforts using cash jars. And to the people of the community who fill them, your "hometown girl" thanks you from the bottom of her little heart.

To our dear friends at **Highlands Presbyterian Church** for their continued love and support of our family and Foundation.

To Kirby's grandmother **Joanne Wilson** for her donation to the Foundation in memory of **Joseph Crawford**.

To **Lynn Regnery** for her donation in memory of **Ryan Topham**.

To the many friends and family of Foundation board member **Kathi Brummet**, who donated in fond memory of her father, **Edward Flannery**.

And To The Families Working Together For The Cure....

To the **IPA Wives Club** for its donation in honor of **Brooke and Ashleigh Kidwell** of Lexington, KY.

To **Amanda Jean Runser** for her contribution in honor of her nephew, **Brandon Joseph Mohr**.

To the Jersey Shore AYSO soccer team, the **Sunbirds**, for its contribution in honor of **Hunter and Sydney Moff** of Williamsport, PA.

To **Mr. & Mrs. Michael Zahra** for their contribution in honor of **Grace Bellontine** of South Huntington, NY.

Steve Moff's "Race for the Cure"

Steve Moff, of Williamsport, PA, father of Sydney and Hunter, who both are afflicted with Sanfilippo, crossed the finish line of the Marine Corps Marathon this past fall with flying colors. Steve chose to personalize his efforts in this marathon by encouraging donations to the Foundation. We are sure he was thinking of his children with pride and their battle with Sanfilippo as he crossed the finish line, but we hope he was proud of himself

as well. Friends, neighbors and business associates raised more than \$18,900 for research. Way to fly, Steve!

News From Connecticut

Totals are in for last fall's annual "Rockin' for Rhianna" benefit dance, organized by **Alan and Donna Theriault** and their group of dedicated volunteers. More than \$17,400 was raised for the Foundation. This is the eighth year for the Theriaults, who heard about a little girl in their community named Rhianna who needed help. Gene and Cynthia Logan are forever grateful for the compassion Alan, Donna and others throughout their community have shown for their daughter over the years.

Mary Couture, Rhianna's aunt, expanded the success of "Rockin' for Rhianna" by contacting the Paul Newman Foundation. The reward was very generous, as Mr. Newman met her request with a \$5,000 donation.



Rhianna with her proud Dad.

FUNDRAISING NEWS

And, the community's efforts continue...

In October of last year, **Kelly Massucci**, Executive Director of **The Talcott Center for Child Development** and Rhianna's occupational therapist, participated in the 5K portion of the Greater Hartford Marathon with four other Talcott staff members. "Running for Rhianna," this small team raised \$555 for research. Preparations are already underway for this year's run, as the Talcott Team hopes to continue and expand its "race to find the cure."

Rochelle and her parents, **Michael and Janice Maiorca**, chose to share the celebration of Rochelle's wedding day with the Foundation. In lieu of table favors, a donation was made to the Foundation in honor of Rhianna. Our best wishes to the newlyweds!

Karen Schick, a special education teacher at the **Vogel-Wetmore School** in Torrington, CT, shared Rhianna's story with her students,

who in turn gathered funds for a donation to the Foundation and created some special cards for Rhianna. The children's response was a very touching story for the Logans.

Memorials were received by the Foundation in memory of **Richard Brown, Sr.** of Plainville from many friends and family members who were so fortunate to know him. Mr. Brown was the father of Cynthia Logan's longtime friend, Laura Schaefer.

Memorials also were received by the Foundation in memory of **Thomas Perniciaro**, owner of Nutrition Kingdom in Plainville, who was always very supportive of the Logans and their efforts to keep Rhianna healthy.

The Artisan's Marketplace holiday fundraiser continues to grow in popularity. The "Santa for Hope" raffle and "Stars for Hope" made more than \$1,700 for the Foundation. "Stars for Hope" is a craft kit for children put together

right at the shop by Rhianna's family and her friend, **Anna Huey**. Customers purchase the kit, allowing their children to create the star and keep it, reminding them of Rhianna. Or they can return the star to the Artisan's Marketplace, where others can purchase it, doubling the donation to the Foundation. Cynthia reports that not only have other children asked to help put together the kits this year, but also customers are already asking for this year's kit! What a shining example of compassion.

And, as always, our thanks to Cory and Donna Gabel, Rhianna's aunt and uncle, for their ongoing donations of a portion of the proceeds from Cory's solo piano instrumental CD, "One Road," to the Foundation. Sales continue, as do donations to the Foundation. Interested in this new "spin" to help? Just contact the Foundation to obtain a copy or log on to Cory's website at cdonavan.com.

RESEARCH UPDATE

Robert K. Yu, Ph.D., Med.Sc.D Institute of Molecular Medicine and Genetics, Medical College of Georgia

Dr. Robert Yu and his team of scientists are using neural stem cells (NSCs) that can be transplanted into brain to correct some of the metabolic defects in Sanfilippo disease (MPSIII).

Dr. Yu: Stem cells are self-renewable and are capable of differentiating into several types of nerve cells, such as neurons and glial cells. Our goal is first to transplant these cells into the brains of animal models with MPS to see if they can correct the storage of heparan sulfate by serving as a source of the corrective enzyme to other neighboring cells in the

brain. We are first studying the biological properties of these cells in test tubes, and in the brain. This basic knowledge is essential because we want to make sure that we can produce enough corrective cells for transplantation, and once they're transplanted, we want to make sure the donor cells survive, migrate, and grow normally within the recipient's brain, in order to

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RESEARCH UPDATE

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avoid abnormal growth of these cells, e.g., tumor growth.

We plan to determine which of these cells serve as the best donors for this sort of transplantation therapy. Once we obtain the desired cells, we will transplant them into animals with MPSIII and examine the effects on the reduction of the stored materials and the long-term consequences. This strategy not only depends on getting a sufficient number of donor cells to survive and grow after transplantation, but also on the levels of corrective enzyme they are able to produce. We are determining whether these corrective cells are capable of making sufficient amounts of the corrective enzyme, but we have also been engineering these cells to give them a boost to their production of corrective enzyme to improve their therapeutic value. Together, these approaches hold a great potential toward achieving an effective therapy for Sanfilippo disease.

**Svitlana Garbuzova-Davis,
Ph.D., D.Sc.
Center for Aging & Brain
Repair, Department of
Neurosurgery
University of South Florida**

During the last few years, our research group, led by Professor Paul R. Sanberg, University of South Florida, has studied the

potential of human umbilical cord blood (hUCB) stem cells to treat neurodegenerative disorders, as well as brain and spinal cord injuries. Data suggest that these cells are able to differentiate into pre-neural cells, making repair of a damaged or defective nervous system possible.

In Sanfilippo syndrome type B, a deficiency of the *Naglu* enzyme leads to accumulation of heparan sulfate, a glycosaminoglycan (GAG). Our results demonstrate that intravenous administration of hUCB cells into *Naglu* mice at different stages of disease (early symptomatic or late stage) has a beneficial effect, probably due to enzyme delivery into these enzyme-deficient mutant mice. Previously shown behavioral improvements, indicating advantages of administered hUCB cells, are supported by results of our current investigation on survival, distribution, and differentiation of the transplanted cells.

The intravenously administrated hUCB cells were found widely distributed inside the central nervous system (CNS) and throughout the body. Although many hUCB cells were found in blood vessels, confirming their continued presence in the blood circulation, some cells were found in tissues of the brain and other organs. Moreover, the migration of cells to organs within the CNS showed that some cells can

develop into neural types.

Importantly, an increase in anti-inflammatory proteins was determined at 6 months post-transplant. Additionally, an obvious reduction of GAG levels was observed in both animal groups at 3 and 6 months after hUCB transplantation. Moreover, heparan sulfate levels in the liver and spleen of 6 months post-transplant mice were significantly reduced. Still, sex-based differences in peripheral blood and increased monocyte levels in peripheral blood need further investigation to better evaluate hUCB cell benefits. It is our suggestion that monocyte/macrophage cord blood cells may aid GAG clearance in this heparan-sulfate-rich environment and may have additional benefit.

The administration of hUCB cells into the systemic circulation of mutant mice may lead to the development of a new strategy for enzyme replacement for Sanfilippo.

However, the ideal hUCB-derived cell population for removing accumulated heparan sulfate is still unclear and remains a critical point in developing our cell transplant strategy.

FUNDRAISING OPPORTUNITIES

A Match For Kirby

Does your company have a matching gift program? It could double your support of the Foundation.

Our thanks to **Steven Kastenholz and Bank of America.**

United Way Can Be For Kirby, Too

Does your company have United Way pledges at your workplace? Although we are not a United Way member, you can designate The Children's Medical Research Foundation as your recipient, and the funds will be forwarded to us through the United Way Campaign! Our United Way Chicago "Agency Number" is 3025558. For those of you out of state wishing to participate, please supply your local United Way

agency with the Foundation name, address and our Federal ID #36-4033667.

Our thanks to **the employees of UPS of Los Angeles, CA, UPS of Lexington, KY, Deerfield High School District #113, University of Chicago Hospitals and SBC.**

Celebrate, And Make It for Kirby

Is there a special birthday coming up for a family member or friend? Are you looking for an alternative to the typical "over the hill" gift? Be different. In lieu of gifts, donate to The Children's Medical Research Foundation. Kirby always loves a party!

A Gift Like No Other

This holiday season give clients a donation to The Children's

Medical Research Foundation in their name. It's a gift that won't gather dust and goes far beyond any other.

Go Kasual for Kirby

Tired of wearing those heels, hose or ties five days a week? Why not suggest a "Go Kasual for Kirby Day" to your employer? It's a great way to give a "relaxing feel" to a workday and raise funds for the Foundation. Simply send a memo to co-workers explaining the day and set the "fee" to participate.

Still in school but want to help? How about a "Hat Day" done the same way! It's fun for the kids and a great way for them to participate in a good cause. No "fee," just leave an amount up to them.

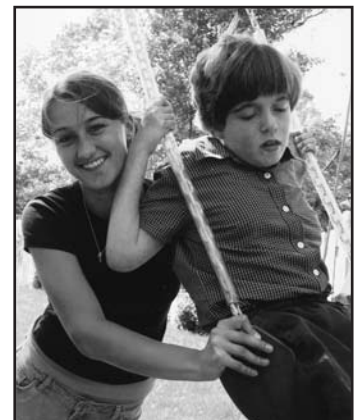
We Need A Boost

Donations to the Foundation have been below levels of past years. Please consider donating to ensure the Foundation is able to continue its "push for a cure" and fund all the research projects that show such great promise.

**Give Kirby a boost!
Your generosity will make all the difference.**

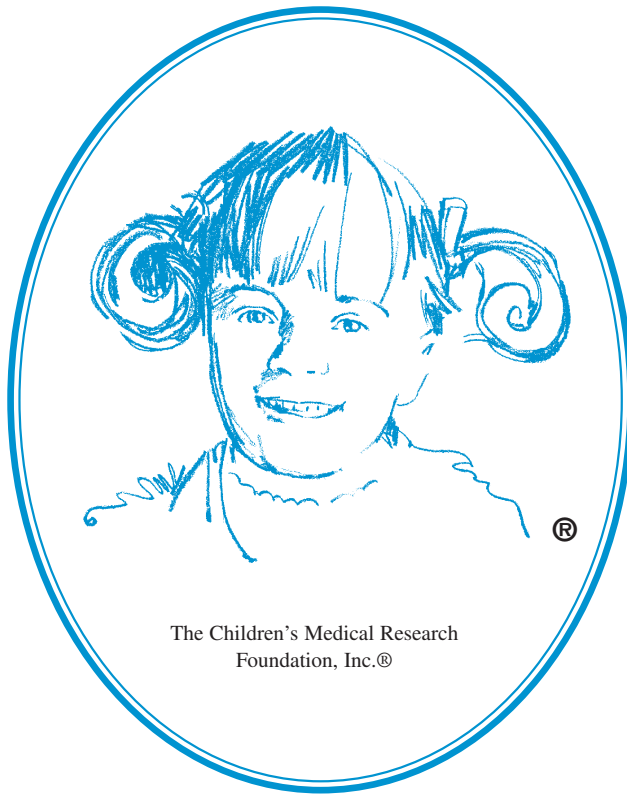
Please send donations now to:
**The Children's Medical Research Foundation, Inc.
P. O. Box 70
Western Springs, IL 60558**

Kirby and all the children afflicted with Sanfilippo thank you.



Kirby gets a boost from her sister Maggie.

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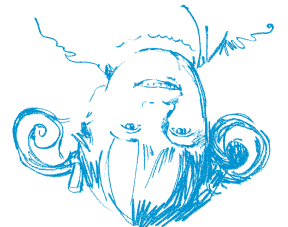


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