



KirbyGram

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

December 2006

Our goal is to create awareness of Sanfilippo Syndrome and other neuro-genetic disorders, fund medical research and find a cure.



Our teacher, April, 2005

Kirby Update

It is hard to believe how fast time goes by, for this fall Kirby started at Lyons Township High School as a freshman! Unfortunately, fall also marked the beginning of her latest struggle with Sanfilippo. Kirby fought all summer to maintain both her mental awareness and ability to walk. By the end of August, we noticed not only the steep decline she had taken in these areas but also what we now know to be uncontrolled muscle spasms. Kirby's anxiety and discomfort from these constant spasms quickly became apparent.

As we continue to work with her doctor to find what we hope will be a balance between the medications available to provide relief and allowing her to maintain some semblance of awareness and mental abilities, she is blessed by the team that surrounds her each day at school. From the first day, teacher Mike Detzner, classroom aides and therapists all have spent time getting to know their new little student and her abilities, while sharing their expertise as they attempt to help with her challenges. All have done nothing but welcome her with open arms and stand ready and willing to do whatever is necessary for her to be the best she can be. It is a joy and a blessing to know people such as this who recognize the viability of children like Kirby and celebrate their lives.

Dear Friends,

The Kirby Update explains how Kirby continues to be challenged both mentally and physically. As Brad and I watch her struggles, we are reminded of some of the most important things she has taught us. The time has come to show her we were listening. Throughout the years, Kirby has met the challenges of this devastating disease with incredible fortitude and courage, always finding happiness and a reason to smile each day. She brings her family and all so fortunate to know her nothing but joy.



As Brad and I face what we believe to be some of the hardest times parents can face, watching a loving child endure seemingly unrelenting distress, we must show her the same fortitude and courage she has shown us as our teacher over the past 15 years. For she still remains resilient and very capable of feeling our emotions. We will never give up in our efforts to bring her comfort and peace. And, we will do it with the same joy she has given us each and every day of her life.

This holiday season, Brad and I wish for you the same — comfort, peace and joy. We ask that you think of Kirby with a smile and in her honor, continue to support The Children's Medical Research Foundation, its mission and the researchers who are working so diligently to find a cure.

May the blessings of this holiday season be yours, now and always.

Brad and Sue Wilson

Fundraising News



“Fore” Kirby

The Eleventh Annual “Fore Kirby” Golf Fun Raiser was held June 2nd with 16 participants. This year’s event was proof of the saying “size doesn’t matter,” as it was the smallest group of golfers to participate in the outing, yet this determined group raised almost \$33,000! Nanci Makris and The Flame of Countryside once again provided refreshments and a delicious dinner buffet at the Wilson’s home after the event.

Sue comments, “Brad and I are so very fortunate to have such dear friends and family members willing to do whatever is asked of them to raise funds and awareness. Each year, this event gives us the opportunity to be with each of them, enjoying their friendship and remembering just how privileged we are to call them our friends and family.”

The Foundation Gives Thanks....

To **Randy and Barbara Bolduc** for their donations in memory of **Jack Palmer and Ginny Longstreet**,

To **The Marmon Group, Inc.** in Chicago and **HSBC** in Princeton, NJ, for their matching gift donations. A perfect match for Kirby!

To **Alison Credit, Margaret Dawe, Colleen Begg, Keith Sampson, Nicholas Megofna, Robert Acorn, Frank Lococo, Jeffrey Proost** and the anonymous donors who designated the Foundation as their charity of choice in their various employers’ United Way campaigns. We appreciate them uniting for Kirby!

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 and have helped to raise more than \$700 to date this year, your “hometown girl” thanks you from the bottom of her little heart, and

To the **LaGrange Highlands Presbyterian Church** and its members for their continued support of the Foundation, as well as the Wilsons. Sue says, “Our church family has been an incredible part of our emotional support from the beginning of this journey, and we are truly blessed by this friendship.” A note recently was received from church treasurer Mark Scheppler, saying “...the check is for \$1,473 and represents year-to-date collections raised by our Church on behalf of your Foundation. I’m sure I speak for many at the Church when I urge you to keep fighting for Kirby and other

children with similar problems. You are an inspiration to us all, and may God bless you and the researchers that you fund.”

Also received from the church was a check from the **Rev. Rounce Memorial Fund**. Rev. Robert Rounce was the pastor of the church for many years and passed away in 2001. He and his wife Ellie’s compassion and friendship meant the world to the Wilson family. Mark Scheppler’s note explained, “I know Bob cared deeply for your family and that he strongly supported the excellent works that have been accomplished through your tireless efforts...he would feel greatly honored that the monies given in his memory were going to a cause that was so dear to him.”

Sue responds, “It was a wonderful moment for Brad and me to not only read Mark’s kind words of support on behalf of the church membership, but also to learn that Ellie had requested that the Foundation receive this donation from the memorial fund. The members of our church and Ellie continue to remind us of the extraordinary people that have come into our lives because of our dear daughter, Kirby. We have much to be thankful for.”



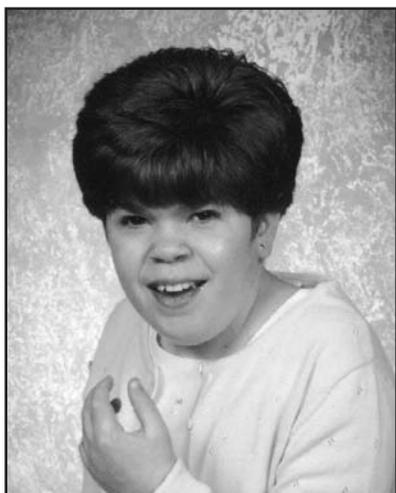


Fundraising News

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



Brooke Kidwell



Ashleigh Kidwell

To **Arthur and Roberta Kidwell**, proud grandparents of **Brooke and Ashleigh Kidwell** of Louisville, KY, for their donation to the Foundation in celebration of Brooke's 14th birthday!

To **John and Joan Bellontine** of Huntington Station, NY, who held their first fundraiser in



Grace Bellontine making a splash.

honor of their five-year-old daughter, **Grace**. The Grace for a Cure 5K Run/Walk, held June 17th, was a great success for both the family and research. Joan's employer, **Xerox Corporation** in Woodbury, NY, started their fundraising efforts off with a \$500 donation to the Foundation. After the event, John and Joan sent the Foundation a check for \$6,500, noting it would be "the first of many donations from Grace for a Cure." The Foundation is very grateful for the Bellontine's help.

News From Connecticut

Alan and Donna Theriault, along with **Mary Couture**, once again were instrumental in

organizing the **Rockin' For Rhianna Benefit Dance**, which was held June 10th. This ninth annual event raised almost \$19,000 for the Foundation. As in the past, **Tom Buonocore of Badge Printers of America** generously donated all the printing needs for the event. A special thanks to the Theriaults, Mary Couture and Tom Buonocore for their commitment to the success of our mission.

The Manafort Family Foundation donated \$2,500 from the proceeds of its annual golf outing in honor of Rhianna. Our thanks to longtime supporters Tabitha and Justin Manafort for teeing up their family for "Rhianna's Hope."

(Continued on Page 4)





Fundraising News

(Continued from Page 3)



Rhianna and Donna Theriault hangin' out in 1999.

Harvey Wilson and The Bristol Auto Club donated \$500 in proceeds from the Club's annual "Cruisin' for Rhianna" car show. The Foundation and Logans appreciate Mr. Wilson and the club members' commitment to their "drive" to find a cure for Sanfilippo.

We thank **The Connecticut Classic Chevy Car Club** and **The Hamden Police Department** for choosing the Foundation as one of the lucky

beneficiaries of their Annual Memorial Day Weekend Classic Car Show on the grounds of **Quinnipiac University**. A \$500 donation was received. The Foundation greatly appreciates these groups and the many individuals who joined forces to make the show such a wonderful success for the charitable causes it supports.

A special thanks goes to car enthusiasts **Mr. & Mrs. Leonard Roberts** of the

Clinton S. Roberts Foundation, who attended the show and donated \$2,000 to the Foundation in honor of Rhianna.

A \$1,000 donation was received from **Rodney and Janice Reynolds** of North Granby, CT, in honor of Rhianna "and her struggle with Sanfilippo Syndrome." The Reynolds further explained, "An announcement will be placed on each table at our daughter's wedding that this contribution has been made in lieu of favors. It is our hope it will enlighten others to the very important mission of CMRF and encourage support to continue their work in finding the cure for neuro-genetic disorders." Our thanks to the Reynolds for sharing their family's special day with the Foundation and its mission of a cure for our children. Congratulations to their bride and her groom!

Research Update

From the Laboratory of Dr. Elizabeth F. Neufeld, Department of Biological Chemistry, D. Geffen School of Medicine at UCLA

Work in progress: getting enzyme into the brain.

The major obstacle to treatment of the Sanfilippo

Syndrome by enzyme replacement is the inability of therapeutic enzyme to get into the brain because of the blood-brain barrier (BBB). The BBB is a property of capillaries in

the brain, which form an essentially impermeable barrier to large molecules such as enzymes. However, the capillaries make an exception for molecules that the brain requires. For example, the brain requires iron, and the capillaries have a mechanism



Research Update



for bringing it from the blood to the brain. Specifically, the iron is bound to transferrin (a protein that carries iron), which in turn binds to transferrin receptor molecules on the blood side of the capillaries, shuttles with the receptor across the capillary and is released on the brain side. This mechanism of transport from one side of the capillary cells to the other is called "transcytosis." Our strategy is to modify lysosomal enzymes so that they can bind to the transferrin receptor and be ferried by transcytosis across the capillary cells into the brain, where they could be taken up by neurons.

We have set out to modify lysosomal enzymes by "aptamers" - synthetic small molecules of nucleic acid (RNA or DNA). Aptamers that bind to any desired molecule can be isolated from very large random mixtures of chemically synthesized RNA or DNA. Thus we have isolated aptamers that bind to the mouse transferrin receptor. We can attach these to model proteins (for example, streptavidin or albumin) and show that such molecules can be taken up by mouse cells in tissue culture. Our next goal is to determine whether aptamer-modified α -N-acetylglucosaminidase,

administered intravenously to MPS IIIB mice, can be delivered to the brain and correct the pathology that is specific to the disease. This study is funded in part by the National Institutes of Health and in part by the Children's Medical Research Foundation.

Work recently completed: characterization of some storage products in the brain of the MPS III B mouse.

In 1999, we had generated a mouse model of MPS IIIB by disrupting the *Naglu* gene, which encodes the enzyme α -N-acetylglucosaminidase. We had found that heparan sulfate, a mucopolysaccharide which cannot be degraded for lack of the enzyme, was stored in excess in many tissues of the MPS IIIB mouse, but rather surprisingly, relatively little heparan sulfate was stored in the brain. We have now found that storage of heparan sulfate was greatest in specific areas of the brain. The areas storing heparan sulfate also accumulated material that does not require α -N-acetylglucosaminidase for degradation. This other material includes the lipids cholesterol and GM3 ganglioside, and the proteins ubiquitin and SCMAS. Storage of these substances

starts when the animals are very young and increases as they age. It is not unique to MPS III B but occurs, to a greater or lesser extent, in many other lysosomal storage diseases. Our interpretation is that the primary storage material (heparan sulfate in the case of MPS III B) causes lysosomes to become greatly enlarged and more numerous, which in turn must generate many signals to which the cell responds, by unknown pathways, with secondary accumulation of these other substances. We don't know how these secondary accumulations affect brain function, but we can use them as "markers" when studying the progression of the disease and the effect of therapy in animal models.

This study was funded by the National Institutes of Health and the Children's Medical Research Foundation.





Fundraising Opportunities

United Way Can Be for Kirby, Too

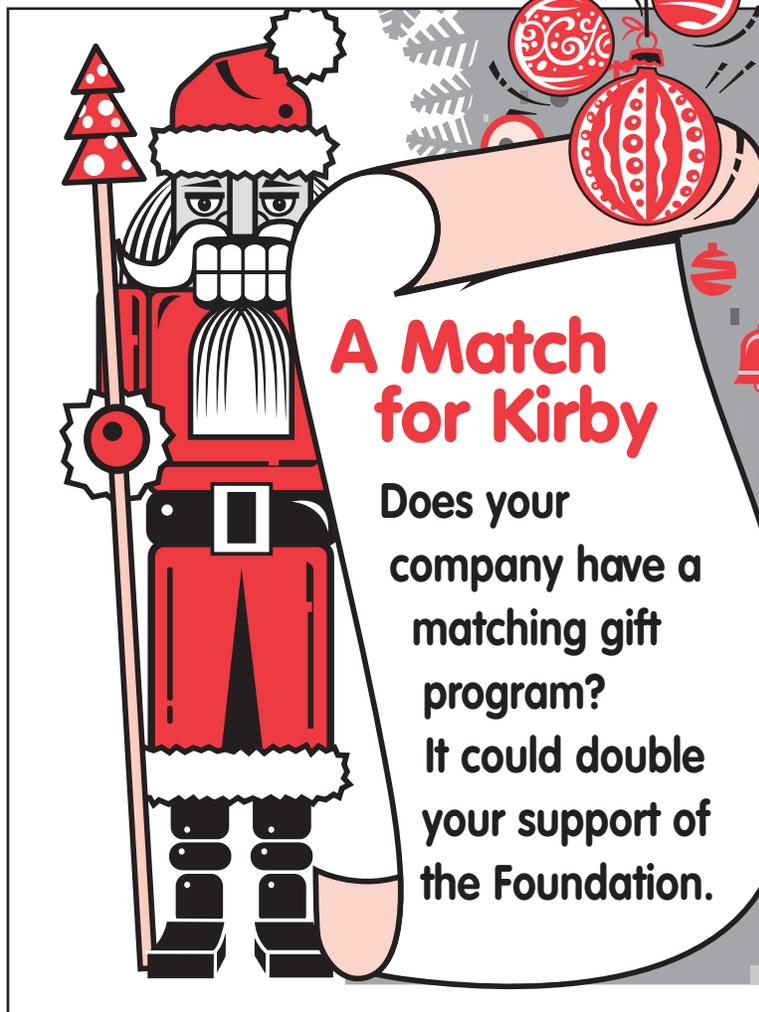
Does your company have United Way pledges at your workplace? Although we are not a United Way member, you can designate The Children's Medical Research Foundation as your recipient, and the funds will be forwarded to us through the United Way Campaign! Simply give your local United Way agency the Foundation name, address and our Federal ID #36-4033667.

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.





The Sweetheart Dinner Dance

February 9, 2007 • The Ritz-Carlton Chicago

An elegant new setting at one of the world's finest hotels

The 12th Annual Sweetheart Dinner Dance will be held Friday, February 9, 2007, in the Ballroom of The Ritz-Carlton Chicago.

We're "Puttin' on the Ritz" with a new venue, a new menu and an all-new exciting raffle.

Executive Chef Mark Payne will serve a signature four-course dinner from his award-winning kitchen, following a cocktail reception in the adjoining Loge.

The dance floor will percolate with music by the renowned Michael Lerich Orchestra. And raffle packages promise to tempt sports, travel, dining, spa and shopping enthusiasts.

Reservations are \$225 per person or \$2,250 for a table of ten and must be made in advance. Invitations will be mailed in December. Please plan to join us by marking your calendars now.

We need your help to make it a success

This dinner dance is the primary fundraising event of the year, and we're asking you to help ensure its success.

We have designated five sponsorship levels for the Sweetheart Dinner Dance, as listed below. In appreciation of your sponsorship, you will receive prominent event recognition. We ask that you indicate your wishes on the following Reply Form and return it to the Foundation. Please contact Sue Wilson at 708-784-0631 with any questions.

The Twelfth Annual Sweetheart Dinner Dance

~ SPONSORSHIP REPLY FORM ~

- Sponsorship:**
- | | | | |
|-----------------------------------|-----------|-------------------------------------|----------|
| <input type="checkbox"/> Diamond | \$ 10,000 | <input type="checkbox"/> Silver | \$ 1,000 |
| <input type="checkbox"/> Platinum | \$ 5,000 | <input type="checkbox"/> Sweetheart | \$ 500 |
| <input type="checkbox"/> Gold | \$ 3,000 | | |

Name as you want it to appear (Please print): _____

Check Enclosed

Please charge my (*check one*) Visa/MC American Express

Cardholder Name (print) _____

Account Number _____

Expiration Date _____ Signature _____

Please return this form to:

*The Children's Medical Research Foundation, Inc., P. O. Box 70, Western Springs, IL 60558,
fax to (708) 784-1978 or call (708) 784-0631.*

Please include your phone number: _____





Save The Date

February 9, 2006

The Sweetheart Dinner Dance Makes a Romantic Holiday Gift

This holiday season, give your sweetheart a gift of good cheer – a romantic evening at the Sweetheart Dinner Dance.

You'll avoid last minute shopping in crowded stores when you call 708-784-0631 now for reservations.

Then enjoy February 9th in taffeta and tux, sipping champagne, dining in the Ballroom of The Ritz-Carlton, and dancing to the music of the Michael Lerich Orchestra. Cheers!

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