The Children's
Medical Research
Foundation, Inc.®

KirbyGram®

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

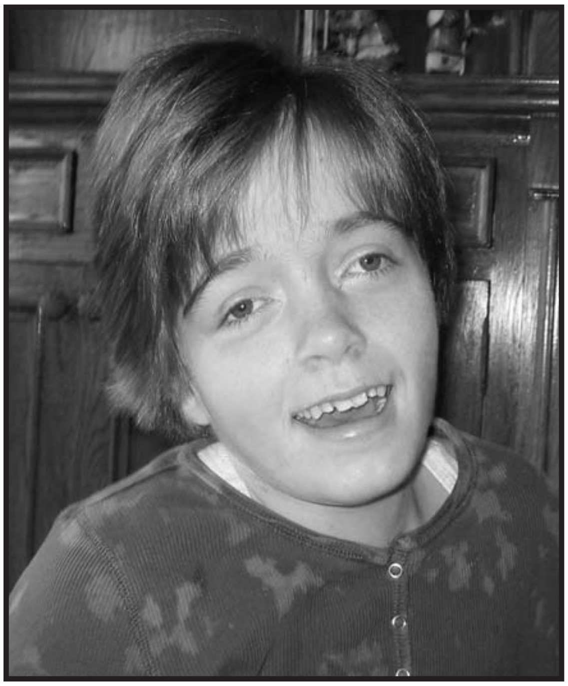
December 2008

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

Kirby Update

It's hard to believe that Kirby is 17 years old and is well into her third year at Lyons Township High School. Classroom teacher Mike Detzner and staff members within the room continue to provide an outstanding environment for her and her fellow students as they have for the past three years.

Sue explains, "Kirby's room is filled with people who believe in their students, celebrate their abilities, nurture their strengths and bring innovative ideas to confront their challenges with the dignity they so richly deserve. Brad and I are grateful to each and every person within the C115 classroom."



Dear Friends,

As Kirby's journey continues, Brad and I have just begun to grasp the amazing grace she demonstrates each day as she defies the manifestations of her disease and reveals to us the power of her soul. Her challenges remain such that it is difficult for us to watch, much less begin to comprehend, what she faces daily. Yet the comfort felt from the gentle grasp of her hand and the joy that shines from within her beautiful blue eyes bring peace to our hearts. She is and will always be our hero.

This holiday season, Brad and I wish for your families and you the same comfort and joy Kirby brings to our lives each day. We ask that you think of Kirby, 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 the season be yours, now and always.

Brad and Sue Wilson

Fundraising News

"Fore" Kirby

The Thirteenth Annual "Fore" Kirby Golf Fun Raiser was held May 30th at Ruffled Feathers Golf Club in Lemont. This year, 20 participants raised more than $34,000 "fore" Kirby!

Sue comments, "Brad and I love this event, as it gives us the opportunity to be with many of our friends and celebrate not only their extraordinary fundraising abilities and enduring support of the Foundation but also their friendship. We also have the pleasure of meeting new people each year who have heard of our daughter, the Foundation, and its mission and have felt it worth their time and effort. It's a great feel-good day for us and a wonderful win for the Foundation!

The Foundation is actively seeking new participants for this unique golf outing. The event is held each year on one of the first Fridays of summer. Participants are given pledge cards and are asked to secure pledges from friends and associates for each of the 18 holes of golf to be played.

Upon completion of the round, score cards are collected from each player, and the Foundation then contacts all of those who have pledged with the results of their player's round and the total amount due. Participants also are asked to pay for their round of golf, which means that 100% of the donations go directly to the Foundation! Cocktails and dinner are served immediately following at the Wilsons' home.

Interested in joining the fun? Please contact Sue at (708)784-0631 to learn more.

The Foundation Gives Thanks….

To the many donors who contributed in memory of **Harriet Notarus**, mother of Foundation board member **Charlie Notarus**.

To **Mr.** and **Mrs. Alfred Angeli** for their donation in memory of **Howard Carne**.

To **Greg** and **Eileen Meyer** for their donation in celebration of Kirby.

To the **LaGrange Highlands Presbyterian Church** and its members for their continued support of the Foundation, as well as of the Wilsons. Sue says, "This church and its members have been a part of our lives since Kirby's diagnosis more than 13 years ago. We are blessed by their steadfast support of the Foundation, their friendship, compassion and prayers for our family."

To **James D. Angus** and **Greg Meyer** for submitting matching gift applications to their employers, **The Marmon Group** and **The CNA Foundation** that in turn sent matching donations to the Foundation and doubled the support for the Foundation. A great match for Kirby!

To **Margaret Dawe**, **Nicholas Megofna** and the **anonymous donor** from **Adams & Knight** who designated the Foundation as their charity of choice in their employer's United Way campaigns. Thanks for uniting for Kirby!

To the many contributors who used the donation envelopes as an opportunity to give to the Foundation. Donations from our June newsletter totaled $985.

To **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 $500 to date this year, your "hometown girl" thanks you from the bottom of her little heart.

And To The Families Working Together For The Cure….

Grace Bellontine, a happy girl!

John and **Joan Bellontine** of Huntington Station, NY are at it again in honor of their daughter, **Grace**. September 20th marked the **"Grace for a Cure"** second annual 5k

Fundraising News

run/walk that included a raffle, live band, DJ, fireworks and fun for all ages. Outback Restaurant came to cook for the crowd as a delicious contribution to the cause. This year's event was attended by 300 people and raised $18,000. The Bellontines recently announced that they again intend to support the Foundation and its work with a $10,000 check from the proceeds of this event.

Not wanting to stop at just one annual event, Joan Bellontine has a new children's game under development called **I Can Do It! With Grace**. First reviews of this "exciting, effective interactive game" are calling it a winner for kids!

The Bellontines have applied for and are looking forward to soon receiving 501 (c) (3) tax exempt status from the government for Grace for a Cure. The Foundation looks forward to welcoming its new partner in its mission of a cure for Sanfilippo.

To **Les** and **Teri Sheaffer** for their donation in memory of **Joe Langford**.

To **Carol Lawson** and **Grandma** and **Grandpa Kidwell** for their donations in celebration of **Brooke** and **Ashleigh Kidwell**.

To **Barry** & **Nancy Boyer** and **Jimmy** and **Mary Jane Baier** for their donations in honor of **Sydney** and **Hunter Moff**.

News From Connecticut

This makes it year number four for **The Manafort Family Foundations** annual charity golf tournament, held in memory of **James Manafort, Sr.** And, lucky for Rhianna that they again chose to tee up their support for **"Rhianna's Hope,"** with a $2,500 donation to the Foundation.

Over the years, **The Bristol Auto Club** has raised thousands of dollars for research with its annual **"Cruisin' for Rhianna"** classic car show. This year was no exception. The club continued its tradition of a "drive" to find a cure with a $500 donation in Rhianna's honor. Car enthusiasts **Mr.** and **Mrs. Leonard Roberts** of the **Clinton S. Roberts Foundation** also chose to show their support of Rhianna and the Foundation's mission with a $2,000 donation.

Dr. William Petit of **The Petit Family Foundation** supports young people's commitment to community service by honoring a few each year with a donation to the charity of their choice. Rhianna's friend **Annalicia Huey** was selected this year as Dr. Petit felt she reminded him of his own daughter, who always supported the underdog. Anna chose the Foundation as the recipient of Dr. Petit's generosity in honor of his family, which was tragically killed last year.

Debra Dawson, one of the original founders of Rhianna's Hope and the first cut-a-thon, along with her sister, **Lee Longo** of **The Hair Spa & Kids Cut** in Plainville, held a **Kids Cut Carnival** in honor of Rhianna and raised $300 for the Foundation. Now that's the kind of cut we can celebrate!

Congratulations go out to **Shannon Byrne** and her new husband **Guy Winter**, on their wedding day this past summer. Shannon, who has enjoyed spending time with her friend, Rhianna always felt they had a special bond and felt Rhianna communicated with her in a very different way. She spoke at the reception about their time together and how much she had learned from Rhianna, most special to her was unconditional love. The couple chose to honor their guests with a donation to the Foundation in lieu of table favors. We thank the newlyweds for thinking of Rhianna and other children like her on their very special day.

Family friends and **"Rockin' for Rhianna"** organizers, **Alan** and **Donna Theriault**, as well as Rhianna's grandparents **James** and **Martha Couture**, chose to celebrate Rhianna's 16th birthday with donations to the Foundation. Too sweet!

We thank **Rod** and **Janice Reynolds** for reminding us of the 50th birthday of Rhianna's dad, **Gene** with their donation to the Foundation in honor of his special day.

And, our thanks to **Gloria Brown** and **Picture Fame** in Plainville, CT, for their donations in honor of Rhianna.

Fundraising News

(Continued from Page 3)

A Personal Note from Rhianna's Mom

Dear Friends and Family of Rhianna's Hope,

It has been a year of milestones in our family, as Rhianna's grandparents celebrated their 50th anniversary, Rhianna's dad his 50th birthday and Rhianna her 16th birthday. Milestones make us stop and think about life, about time, about the past, present and future. As I stand in disbelief that Rhianna is now 16, it becomes very real when I think of all she has accomplished. She has inspired so many and touched the lives of others, altering their journey in a profound way. I know I am a very different person because of her. We as a family have met so many wonderful people… exceptional doctors, therapists, other MPS families, especially Sue and Brad Wilson, and all the incredibly caring people who have rallied to support our fundraising efforts to find a cure.

I take this opportunity to say THANK YOU to everyone who has contributed. When it all began, I would send a personal thank you note for each donation. I realized I would not be able to continue to do this as the priority of Rhianna's needs became all-consuming. I am grateful for the years of support that have allowed us to send funding to The Foundation. It is the efforts of the Wilson Family and others like them that have changed the course of science. Funding has increased the number of researchers working toward a cure. Researchers have made remarkable progress and are successful in treating some of the MPS disorders. We were told we would not see this in our lifetime, so HOPE is alive.

Rhianna's 16!

Over the years, my hope has hit the bumps in the road, but as a Mom, you never let go. You always hang on to possibility, and when that weakens, you pray for a miracle. We have been very blessed throughout Rhianna's 16 years, for she has exceeded all expectations and done some very remarkable things with her life. This year our fundraising efforts have diminished for many reasons. We remain incredibly grateful to all who created the major events and for their fortitude to carry on for over 10 years. We greatly appreciate those who continue to send donations. We have learned that even a small amount of money can make a difference. Sadly, Rhianna's needs continue to increase as Sanfilippo takes its toll, putting fundraising on the back burner in our house. That leaves me with more sadness as I think of the Wilsons struggling to care for Kirby and still trying to meet the requests for grants.

Rhianna and friend, Shane.

There is still a need to fund research, a need to find a cure. What has changed for me is the importance of a cure. Yes, that is the ultimate goal and what a gift it would be. However, the daily struggle for Rhianna brings forth the realization that a treatment to bring comfort is a simpler goal and still a most extraordinary gift. Despite her difficulties, Rhianna works diligently to smile, to reach for a hand, to light up when her friends visit and to look directly at those she cares so much about with eyes that say "I love you."

2008 will pass without a major fundraiser for Rhianna's Hope. As the end of every year brings us to the season of giving, I ask that when you think about gift-giving, please consider a donation to The Children's Medical Research Foundation in honor of Rhianna. I can't offer anything in return - no raffle item, no ad, no night of dancing - but I can offer the thought that ***this could be the most impressive gift you ever give.***

Rhianna, Gene and I wish everyone a most joyous holiday and a blessed new year.

With sincere appreciation,
Cynthia Logan

Research Update

Update from the Laboratory of Dr. Elizabeth F. Neufeld, Department of Biological Chemistry at UCLA, November, 2008

Nine years ago, with the help of funding from the Children's Medical Research Foundation, we generated a mouse model of Sanfilippo Syndrome type B (MPS III B). Like patients with MPS III B, the mouse lacks the enzyme a-N-acetylglucosaminidase and is unable to fully break down heparan sulfate; this material therefore accumulates in lysosomes of liver, spleen and many other organs. In the brain, the situation is more complicated. Some neurons accumulate additional substances which are not biochemically related to heparan sulfate or to each other. These include lipids (cholesterol and GM3 ganglioside) and proteins (ubiquitin and SCMAS). These secondary storage materials are not found throughout the brain but are localized to neurons in very specific areas. Their role in the disease process is not known. Furthermore, they can be found in brain of mouse models of other lysosomal storage diseases.

To determine why the neurons accumulate these secondary materials, we compared them to neurons from the same small area in normal mice. The brain area in question is the medial entorhinal cortex (MEC). To be sure that we were studying neurons, we first dissected 500 neurons from the MEC of MPS III B and control mice, two months old, and compared the expression of genes. Over a hundred genes were expressed at significantly different levels in MEC neurons of the mutant mice (either higher or lower than in the control mice), but only one stood out – the gene for the enzyme "lysozyme", which was expressed at a level almost 7 times higher in the mutant than in the control. This was very surprising because lysozyme is known to be important in fighting bacteria and is found in cells specializing in this function; it was not known to be present in neurons. But we confirmed the excess of lysozyme protein in neurons of the MEC of the MPS III B mice by specific staining and visualization under the microscope.

It has been noted in the literature that lysozyme is toxic to neurons and can lead to accumulation of hyperphosphorylated tau, a pathological substance found in the brain of patients with Alzheimer's disease. We therefore looked for hyperphosphorylated tau in the brain of our MPS III B mouse and found it in the MEC area. Furthermore, in older mice (6 months old), we also found inclusions consisting of hyperphosphorylated tau in another (closely associated) area of the brain, the dentate gyrus of the hippocampus. The MEC and dentate gyrus are known to be involved in learning and memory. If such inclusions are also formed in the brains of human patients, they may be the cause of the neurodegeneration seen in the disease. There is only one other lysosomal storage disease, Niemann-Pick type C, in which hyperphosphorylated tau inclusions have been found. We suspect that they are present in many other lysosomal storage diseases, but have been overlooked because they are found only in a very small area of the brain.

The unexpected similarity between some aspects of MPS III B and of Alzheimer's disease means that investigators in MPS III B will be able to tap into the vast amount of research conducted on Alzheimer's disease, including the search for drugs that prevent the accumulation of hyperphosphorylated tau.

Fundraising Opportunities

United Way Can Be For Kirby, Too

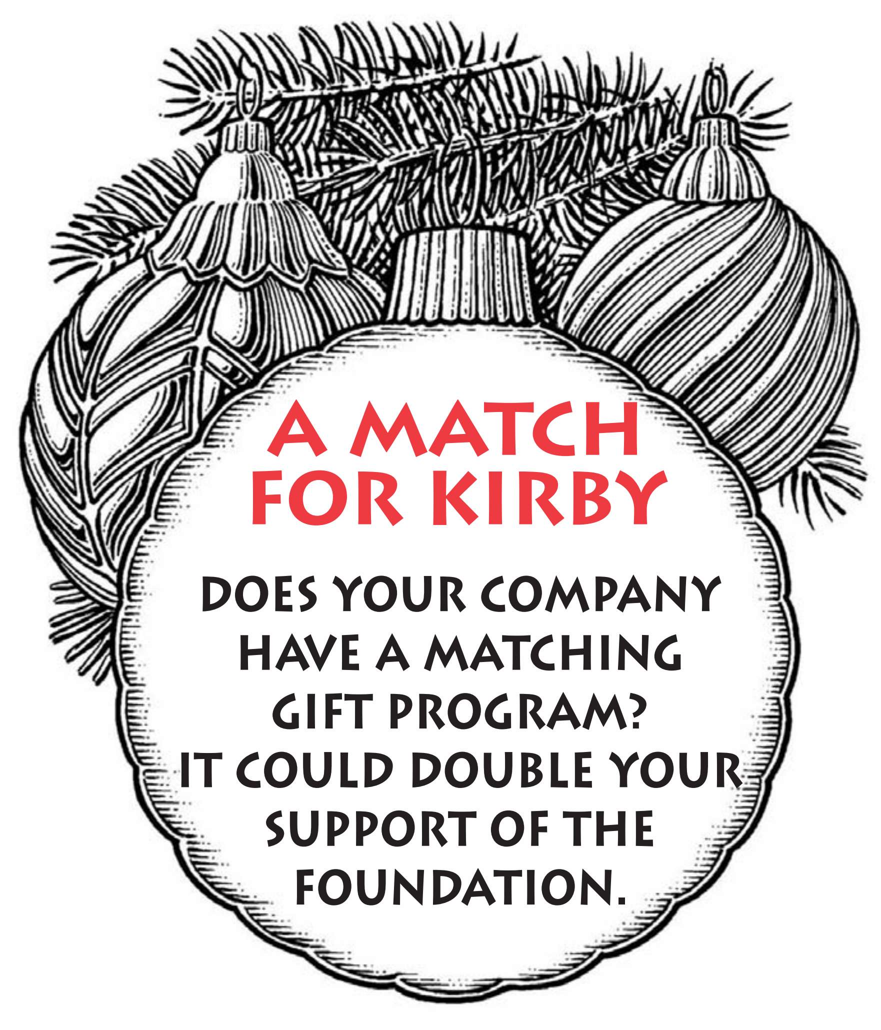
Does your company have United Way pledges at your workplace? Although we are not a United Way member, you can designate The Children's Medical Research Foundation as your recipient, and the funds will be forwarded to us through the United Way Campaign! Simply give your local United Way agency the Foundation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

Give Kirby Security

Tired of taxes? The Foundation now has a brokerage account available that allows you to donate appreciated securities. Why pay tax on the gains when you can realize a charitable deduction of the full market value of your stocks…and it's for Kirby, too! Contact Sue Wilson at (708) 784-0631 to learn more.

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 MATCH FOR KIRBY

DOES YOUR COMPANY HAVE A MATCHING GIFT PROGRAM? IT COULD DOUBLE YOUR SUPPORT OF THE FOUNDATION.

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 6, 2009 • THE RITZ-CARLTON CHICAGO

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

The 14th Annual Sweetheart Dinner Dance will be held Friday, February 6, 2009, in the Ballroom of The Ritz-Carlton Chicago.

We're "Puttin' on the Ritz" at this elegant venue with a new menu and an exciting live auction hosted by Greg "G Man" Dellinger.

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

Guests will be treated to the magical sounds of the Michael Lerich Orchestra, which has entertained us for the past 13 years! And live auction 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 14th Annual Sweetheart Dinner Dance

~ SPONSORSHIP REPLY FORM ~

Sponsorship:	☐ **Diamond**	**$10,000**	Please charge my *(check one)*
	☐ **Platinum**	**$ 5,000**	
	☐ **Gold**	**$ 3,000**	☐ Visa/MC
	☐ **Silver**	**$ 1,000**	
	☐ **Sweetheart**	**$ 500**	☐ American Express

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

☐ Check Enclosed

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 6, 2009

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 6th 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!*

KirbyGram

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