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 2014

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

Dear Friends,

Next year will mark our 20th year of the Foundation. Reflecting is bittersweet. First thoughts take Brad and me back to when we learned that heartache is painfully real, not just a word. Our four-year-old bundle of joy was diagnosed with Sanfilippo, and thoughts of this reality and what the future might hold brought us to our knees. But very soon after that, all of you learned of Kirby and the hopes of our family that a cure could be found. Your overwhelming response of compassion and confidence defined our mission - A Cure for Kirby. So our journey began.

Over the years, Kirby's seemingly endless resolve to find joy amidst her daily adversities gave us our strength. Kirby learned to sight-read names, count, kick a soccer ball, throw her basketball in the "poop," ride a bike, slide and swing. And then there was her singing - our cherished memory.

All the while, you were there with your unwavering resolve to find the cure. And the accomplishments you have helped to enable are many: the expansion of Sanfilippo research in the United States and abroad; decoding the missing enzyme; the development of a mouse model; its inclusion in all the most promising areas of research - gene therapy, enzyme replacement, pharmaceutical and stem cell - and now, bringing research from the laboratory to patients – a human trial.

But times have changed for Kirby. She is now totally dependent in all areas of care and spends her days with us. We are grateful for her comfort and find joy in having her by our sides. We are keenly aware of this gift of time. Our role as parents has never been more defined. It is for this reason we say that the upcoming Sweetheart Dinner Dance will be our last fundraiser of its kind, not because we are done, but because of our need for personal time with Kirby.

The Foundation's mission, too, has never been more defined -- the advances achieved, the real hope of a cure – if not for Kirby, then in her honor. We must find a way. We are sure of Kirby's joy in this for others who have gone before her and for a future for those just starting to show their families the same true joyful moments we are blessed to have as memories.

Times change for all of us, and as our daughter Maggie first said upon learning of her sister's diagnosis, "Kirby will be different?" Yes, Maggie. "That's okay Mom, different can be fun." So please join us one last time in taffeta and tux. Raise a glass and give a toast to our hopes of Cheers in our 20th Year and think about the different fun yet to come.

We wish you comfort and joy this holiday season and beyond,
Sue and Brad Wilson

A year of comfort and joy.

Pucker Up!

Kirby's sister, Maggie, has a challenge for you and it's a sweet one! It's #KissesForKirby. We already know you'd do just about anything for the ones you love, so what about sharing your best kiss photo to raise awareness of Sanfilippo. Share your photos using #KissesForKirby on Facebook, Twitter, or Instagram. Your photo not only will show your love for a cure, but also will be entered to win two reservations for the February 13, 2015, Sweetheart Dinner Dance, a $500 value! We think it is too sweet to pass up, so pucker up. Contest will start January 2, 2015, and will continue through and including February 2, 2015. Winner will be notified no later than February 4th.

Please visit www.curekirby.org for Official Rules.

Fundraising News

A Sweet Night

The 19th Annual Sweetheart Dinner Dance was held February 14th at the Four Seasons Hotel Chicago. This Valentine's Day event attracted 138 sweethearts who that enjoyed a champagne cocktail reception, an elegant four-course dinner and entertainment by Michael Lerich's talented group of musicians. Many guests reported that it was the best dinner dance yet! The evening's generous sponsors, along with some enthusiastic bidding in both the live and silent auctions, enabled The Foundation to raise over $61,000.

Sue comments, "The Sweetheart Dinner Dance is a special evening for us, as its loyal guests are a reminder of the compassion and commitment shown to our family and mission. While the economy continues its slow recovery, not-for-profits are still finding it challenging to attract donors to their causes. Yet through these challenges, the Foundation has been able to continue its critical mission of funding researchers and enabling science to advance toward a cure. Brad and I are honored by the depth of these donors' devotion and are blessed by their presence in our lives."

Say Cheers!

Purple Lemonade – A Stand for Sanfilippo Syndrome is the Foundation's latest and ongoing fundraising effort. Along with other family foundations involved in raising funds toward the cure for Sanfilippo Syndrome, we were invited to participate in a national effort called Purple Lemonade – A Stand for Sanfilippo Syndrome. It was the vision of Grey Chapin, a six-year-old determined to fund a cure for the disorder that afflicts her older sister Blair. Gray's idea was that "If a glass of lemonade could save a child – would you buy one? Of course you would!"

Michelle Fink, Kirby's physical therapist at Perform Physical Therapy in Countryside, learned of our efforts. Her children eagerly spread the word throughout their community, and within weeks, July 9th was chosen as the date for nine groups of children to set up their stands and sell lemonade and baked goods to help a little girl they just heard about. That is, till Kirby pulled up to greet and thank them. Sue Wilson comments, "It was a WONDERFUL day to drive to these homes and see children waving signs and enthusiastically putting the hard sell on for Kirby." Sue continues, "And it was heartwarming to see their faces light up, expressions filled with compassion, when they saw Kirby. After listening to me talk with them about her, Sanfilippo and the hopes of a cure, their pride in being a part of helping their new friend was unmistakable."

A main event page (listed below) has been set up for this group marketing

(Continued on Page 3)

Fundraising News

event to inspire people like you to help the group reach its goal of $1 million. We ask for you to follow the links to the Foundation's team page and consider being a part of our team by hosting a live stand, rolling out a virtual one, sharing with family and friends, or simply buy a virtual glass and saying cheers to Grey and the purity of her idea to save her sister and bring attention this devastating disease. To date, over $64,000 has been raised.

Please visit - http://www.theblairbanner.com to learn about the Chapin family and Purple Lemonade.

http://www.theblairbanner.com/events/purple-lemonade/ for more information on hosting a physical lemonade stand.

http://www.crowdrise.com/PurpleLemonadeStand to host a virtual stand, donate, share or to learn more about this community effort.

Stands, whether virtual or live, do NOT have to be elaborate or raise thousands. This is a true "it takes a village" effort and every dollar is greatly appreciated. Credit card donations made through this site do have a $10 minimum, but all dollars raised can be shown on the site, adding to the team total. Build your team by recruiting family, friends and business associates to help with your stand, donate or simply share our story.

When posting/tweeting remember to use #purplelemonadestand.

Please contact Sue Wilson with questions.

The Foundation Gives Thanks . . .

To **Marian Lynch** for her kind words and donation in honor of Kirby.

To **Debra and Robert Howard** and **Danielle Kidwell** for their donations and to their employers **Denbury Resources, Inc.** and **The Humana Foundation** for their matching gifts, which doubled the donations!

To **Donna Logan-Gabel, Margaret Dawe** and **Nicholas Megofna,** who designated the Foundation as their charity of choice in their employers' United Way campaigns. Thanks for uniting for Kirby!

To **Mary Lou Spedale** and **Alexis Feinstein** for designating the Foundation to receive memorials in fond memory of their father, **Sam Spedale**.

To **Barbara Cummings** for her donation in memory of **Reed Hagee.**

To **Kirschbaum's Bakery** and **Casey's Market**, both 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 $490 to date this year, your "hometown girl" thanks you from the bottom of her little heart.

Ashleigh getting a hand from a friend.

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

To **Wendy and Rob Slattery** for their donation toward "A Cure" in fond memory of their son **Andrew Thomas Coffey Slattery.**

To **Marlene and Bruce VanWagner, Judy Bible** and **Roberta and Art Kidwell** for their donations honoring **Ashleigh and Brooke Kidwell**. And to their parents, **Anna and Dave Kidwell**, as they gather their silent auction items in preparation for their annual trip to Chicago to assist the Wilsons and support the Foundation's Annual Sweetheart Dinner Dance in honor of their girls.

Brooke is a happy girl.

To **Theresa and Steve Moff** of Williamsport, PA, parents of **Sydney**

(Continued on Page 4)

Fundraising News

The Moff family - Theresa, Sydney, Stefan, Hunter and Steve.

and Hunter. This year, Steve chose to jump into fundraising, literally! This past October the family traveled to Dana Point, CA, where Steve participated in his first "Battle of the Paddle," a 4-mile ocean paddle board race where he endured collisions with fellow participants in the choppy water conditions but was able to finish the race in honor of his children. Steve wrote about his experience on the family's website www.sydneyandhunter.org. As a lead-up to the race, the Moffs organized a wine-tasting event. To date, the Foundation has received over $12,000 in donations.

Rhianna's Hope continues . . .

Last holiday season's fundraising efforts by **Artisan's Marketplace** raised $590 for the Foundation. This annual event reminds the Logans each year that the fundraiser's name, "Stars for Hope," is not just about ornaments, but also the generosity of the many "stars" within their community who keep Rhianna's Hope shining bright. These "stars" included: **Sharon and Wayne Albertini, Joanne and Peter Brandien, Martha and Jim Couture, Donna and Alan Theriault, Wendell Theriault, Joanna Ouellette, Felicia, Magdalena and Robert Logan, Mr. and Mrs. John St. Pierre, Mr. and Mrs. Robert Nardozzi, The Bristol Auto Club, Roger and Elaine Karabin and Lucy Baker** to name a few!

Cynthia and Gene Logan chose to honor their daughter **Rhianna's** 22nd birthday by creating a team for the Purple Lemonade Stand for Sanfilippo online fundraiser. As Cynthia looks into Rhianna's eyes, she hears her daughter's words…

"On October 15th, 2014, I will celebrate my 22nd birthday. I watch as my family and friends struggle to find something they can give me. There is nothing I need except comfort; there is nothing I want except a cure.

When I was diagnosed with Sanfilippo over 19 years ago, my parents were told that they would not see a treatment in their lifetime and to expect a cure was futile. We as a family were very fortunate that our friends, family and even strangers believed that we could make a difference, so Rhianna's Hope began to raise money to fund medical research. We were also blessed to find Sanfilippo parents Sue and Brad Wilson, who had established The Children's Medical Research Foundation. The money we raised had an avenue to go where it truly could make a difference.

Today my parents and I have a level of gratitude that is hard to express in words. A tremendous amount of kindness and generosity has brought us to this remarkable moment...there is now a timeline to a cure. This is a critical juncture because what has not changed is our race against the clock and the need for funding. I ask that you help me celebrate my birthday with renewed HOPE and continued support of our fundraising efforts.

Sanfilippo is a relentless illness. I continue to fight to meet my challenges but time is my enemy. I have not been able to speak since the age of eight, so you may wonder how my words have made there way to paper - my mom found them in my eyes. Please join us, the Wilsons and the other Sanfilippo foundations as we step up our efforts. There is strength in numbers and a cure is possible!"

https://www.crowdrise.com/purplelemonade4cmrf/fundraiser/RhiannasHope

With heartfelt thanks, Rhianna

Cynthia, Rhianna and Gene Logan.

Research Update

Abeona Therapeutics

Abeona was founded in March 2013 to develop therapies for patients with lysosomal storage diseases, which includes Sanfilippo Syndrome. Abeona has exclusive rights to develop its two lead products, ABX-A and ABX-B, for Sanfilippo based on therapies developed by Drs. Doug McCarty and Haiyan Fu at Nationwide Children's Hospital. Abeona is currently working collaboratively with the doctors and scientists at Nationwide to see the products through the necessary government and manufacturing processes and required testing prior to human trials.

Timothy Miller, Ph.D, is Abeona's President and CEO and provides the following progress points.

The Natural History Study, co-funded with two other family foundations in 2013, has 85% of patients enrolled, with seven patients already through six months of follow-up.

It is anticipated that the Investigational New Drug (IND) application for Sanfilippo B will be submitted to the FDA in November 2014. The IND is required prior to any

(Continued on Page 5)

Research Update

testing of diagnostic or therapeutic potential in humans. The application contains preclinical data to permit an assessment as to whether the product is reasonably safe for initial testing in humans, manufacturing information to ensure production meets regulatory standards, and the clinical trial design. The clinical protocols and investigator information is also required to ensure that the initial-phase trials will not expose subjects to unnecessary risks and that the clinical investigators who will oversee the administration are qualified to fulfill their clinical trial duties. Finally, commitments to obtain informed consents, obtain review of the study by an institutional review board and to adhere to the investigational new drug regulations are required. A response is typically received within 30 days.

Over $4.5 million out of the $6.5 million target has been raised toward the human trials for Sanfilippo patients in the US and Spain.

It is anticipated that enrollment of the first USA patients for both Sanfilippo A and B programs will begin in the first quarter of 2015.

The Wilsons speak in Washington

On March 12th, the Recombinant DNA Advisory Committee (RAC) for the FDA unanimously voted to recommend approval of the Sanfilippo B trial to the FDA. Sue and Brad Wilson were one of the families invited to give an impact statement after Nationwide's presentation and were present to witness the voting. It was a big step forward and a day the Wilsons won't soon forget. Sue explains, "We were grateful, after 19 years, not only to be invited to speak and be present for the vote, but to then be approached by committee members who spoke of the importance of family foundations' work and parents commitment to their children's well being."

The presentation, in its entirety, can be viewed by following the link listed below. Please note: This is a videocast of the entire day of presentations. It is listed as the 137th Recombinant DNA Advisory Committee (RAC) – March 2014. Nationwide's presentation starts at 2:38.36, the impact statements at 3:48.52 and the vote at 4:05. http://videocast.nih.gov/PastEvents.asp?c=91 - See more at: http://www.curekirby.org/breakthrough/#sthash.VMy4uCs5.dpuf

In Memory of . . .

Benjamin Siedman

It is with a heavy heart that we talk about the loss this year of two children afflicted with Sanfilippo.

Benjamin Siedman passed away this past February, just shy of his 18th birthday. Ben's parents, Jennifer and Stuart Seidman of Wellesley, MA, worked with the Foundation for a couple of years before forming "Ben's Dream" in honor of his 5th birthday. Since that time, the Siedmans and Wilsons have worked together to fund research.

Bruce Chapin, grandfather of Blair Chapin, Ben's friend who also bravely faces the challenges of Sanfilippo Syndrome, said it best after learning of Ben's passing.

"Ben's dream was to be a farmer. Not only was Ben the farmer he always wanted to be, he was the best! He was responsible for the planting of the seed of hope – the hope for a cure. A seed valiantly nourished by his family and now by so many others he inspired."

Kimberly Fowler

Kimberly Fowler of Shelby Twp., MI, daughter of Betsy and Stephen Fowler and sister to Jeff, died this past August at the age of 26. The Fowler family is a longtime donor to the Foundation in support of its mission of a cure.

Below are excerpts from Stephen's thoughts on his little girl.

Kimberly Fowler was a very special girl who touched the lives of so many people. She taught us the importance of patience, compassion and most of all unconditional love. She made us focus on all the beautiful things that the Lord has provided. We took nothing for granted.

The most important things in life to us were not our material possessions. It was family. The best days for the Fowlers were the days Kimmie Fowler smiled. And when Kim smiled, it was almost always because of the love and care given to her by Jeff and Betsy. Like Kimmie, they truly are a gift from heaven. Life is good.

…my wish is that everyone realizes how short a time we have here on this earth. Appreciate each day. Be happy, be positive and don't hold grudges. Hug your spouse, hug your kids, appreciate your friends and tell everyone that you love them. Life is a gift.

Stephen Fowler, Father

Fundraising Opportunities

Have Fun With This Office "Fun" Raiser – Kirby Dares You!

Here's a fundraising idea that can be challenging and fun for everyone in your office. Are you willing to take a dare for Kirby? Here's how it works. Your company pledges a total amount it would be willing to donate to the Foundation. Then each employee willing to take on a dare chooses the dare and the amount to be donated if he or she follows through. There can be a list of suggested dares with donation amounts, or you can leave it up to the employees to get creative and have some fun. Do a dance or perform a song on the street, or for the office, wear heels for a day (that would be a guy thing), get a Mohawk, do cartwheels down a hall, eat or drink a mystery concoction….all for fun and a great cause.

A Match For Kirby

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

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 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.

Kirby's Going Green!

We no longer issue the June edition of the *KirbyGram*.
But, you can receive late-breaking news and event updates
while helping the Foundation to go green and
save much-needed funds at the same time.
Please e-mail your name and phone number to curekirby@sbcglobal.net
to help us start a new, greener database of our supporters.

The 20th Annual Sweetheart Dinner Dance

Friday, February 13, 2015

at the

Four Seasons Hotel Chicago

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

The 20th annual Sweetheart Dinner Dance will be held Friday, February 13, 2015, in the Ballroom of the Four Seasons Hotel Chicago.

Guests will be served a four-course dinner in contemporary American style enhanced by the great bounty of the Midwest.

We will be treated to the magical sounds of the Michael Lerich Orchestra, which has entertained us for the past 19 years! And silent auction packages promise to tempt sports, travel, dining, spa and shopping enthusiasts.

Reservations are $250 per person or $2,500 for a table of ten and must be made in advance. Invitations will be mailed in December, or you can reserve your place now online at www.curekirby.org. 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 or visit us online at www.curekirby.org. Please contact Sue Wilson at (708) 784-0631 or curekirby@sbcglobal.net with any questions.

The 20h Annual Sweetheart Dinner Dance

SPONSORSHIP REPLY FORM

Sponsorship:	☐	Diamond	$10,000	☐ Silver	$ 1,000
	☐	Platinum	$ 5,000	☐ Sweetheart	$ 500
	☐	Gold	$ 3,000		

Name as you want it to appear (please print) ____________________

Check Enclosed ____

Please charge my (check one) Disc ____ Visa/MC _____ AMX _____

Cardholder Name (print) ____________________

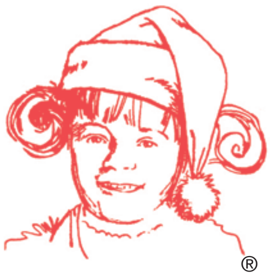
Billing Address ____________________

Account Number ____________________

Expiration Date ____________________ Sec. Code ____________________

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 ____________________



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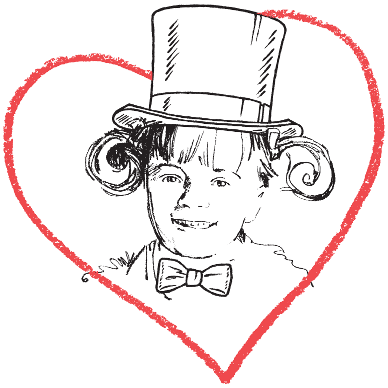

Save the Date

Friday,
February 13, 2015

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 go online (www.curekirby.org) or call (708) 784-0631 now for reservations.

Then enjoy February 13th in taffeta and tux, sipping champagne, dining at the Four Seasons Hotel Chicago and dancing to the music of the Michael Lerich Orchestra. Cheers!