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 2015

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

Years of joyful memories.

Dear Friends,

37. That's the number of times I have written this note to you. Each time my intention was to give you a peek into Kirby's life and express our gratitude for your compassion and your support of this Foundation. What I realize now, in looking back and thinking about my words to you this year, is that these letters are a beautiful chronicle of our life with Kirby and the many joyful memories we are blessed to have experienced with her.

24 years and counting of precious memories. Memories of beach vacations, fun family adventures in the woods, birthday celebrations that included "nic-nics with dog-dogs," (Kirby's own words in expressing picnics with hot dogs) her happy days filled with song, laughter and that beautiful smile - each one just as cherished as the next. Although all the while, we were mindful of the challenges that she faced. These notes are also a testament to her resilience and strength. She, like the many other children with Sanfilippo, shows us the way.

Memories are all some families have left of their children, as Sanfilippo has taken far too many from us. Recently, we were made aware of a family that was faced with the sudden and unexpected loss of their 9-year-old son Landon, who was seemingly doing so well. Now, left with cherished memories, his mother, Ashley Riggs spoke words that were a raw description of grief, and they made our hearts ache. But, what we noticed amidst her words was her strength - strength given to her by her son, his life and his memories. She stated she would never "get over it," that part of her is gone. Even when she is able to find happiness, she will mourn. It will just be a "new normal" as she learns to live again. And, just when I thought her painfully descriptive words would end with "Life is random, unfair and cruel," she continued. "But it is also beautiful and full of amazing things that make the pain and the unbearable ache of loss worth every second. The beautiful memories…are worth any pain, any price." She will use them "to be strong, to move forward" and make him proud and to honor this "precious and amazing" boy.

So we dedicate this to Landon and all the other precious and amazing children who have passed on as we move forward to make the cure the new normal.

2016 holds hope for this with the multiple trials underway and more due to begin. The Foundation will be granting funds to assist in the production of the vector that will be used to treat patients in one of these trials. This is something that we are incredibly proud of. To be a part of the cure from the start to finish will be our honor. And, it is with gratitude we recognize your 21 years of sustaining support that will lead us to this honor. You have empowered our lives as Kirby has inspired and enlightened our journey.

We wish you joy and happiness this holiday season and beyond.

Sue and Brad Wilson

Fundraising News

The Last of the Sweethearts

The 20th Annual Sweetheart Dinner Dance was held February 13th at the Four Seasons Hotel Chicago. The last of the Foundation's signature dinner dances had 144 sweethearts in attendance and raised over $77,000. Guests enjoyed a champagne cocktail reception with the addition of a very special "Kirby Cocktail, " a mini-old fashioned to honor the memory of Kirby's beloved "Grandma." Michael Lerich's talented group of musicians took a final bow after entertaining guests for all 20 years. Sue Wilson comments, "Throughout the years this evening had become such a reflective time for our family that highlighted our many blessings. We will miss the many joyful experiences but realize times have changed and so must our fundraising efforts to see the human trial through to its conclusion."

A couple of sweethearts.

Sue continues, "Many of our guests have attended every Sweetheart. Michael Lerich pledged his commitment to share his wonderful music with our cause the very first year and never skipped a beat. Every single year his musicians had guests begging for just one more dance, not wanting the fun to end. Brad and I are honored by the depth of devotion people have shown to our family, the Foundation and its mission."

Girl Power

The LaGrange Highlands Woman's Club once again chose the Foundation as its Charity of the Year. As the recipient, the Foundation was the major benefactor of the club's entire year of fundraising efforts, highlighted by its annual craft show held each November at the LaGrange Highlands Middle School. A check for $7,700 was presented to Sue Wilson in May at the Club's dinner, which honors its officers and highlights all the year's benevolence recipients. Sue comments, "These woman all live within our community, and I am honored to have them as friends and neighbors. They have never lost faith in our mission, as their steadfast support of the Foundation over the years has shown. "

Sue Wilson with daughter Maggie.

LTHS Student Council Votes for Kirby

Lyons Township High School's 2014-15 Student Council chose to support the Foundation after listening to Sue Wilson talk of the Foundation, its mission and their former classmate, Kirby. A check for $5,040 was received in June as a result of the council's fundraising efforts throughout the school year. Sue explains, "I have nothing but fond memories and gratitude for the staff and students who were a part of Kirby's high school experience. Over the years, they stood ready and willing to do whatever was necessary for her to be the best she could be. To be invited back to speak was exciting. But, being chosen and realizing the students' compassion for Kirby continues, as does their hopes of a cure, is humbling and inspiring."

Say Cheers!

Purple Lemonade – A Stand for Sanfilippo Syndrome is the Foundation's 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. Grey's idea was that "If a glass of lemonade could save a child – would you buy one? Of course you would!"

The Logan family's supporters of Plainville, CT, put a winter twist on the idea and sold hot chocolate, raising $690 in honor of their hometown sweetheart, Rhianna Logan.

A main event page (listed on the following page) has been set up for this group marketing 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 and sharing with family and friends, or simply buying a virtual glass and saying cheers to Grey and the purity of her idea to save her sister and bring attention to this devastating disease. To date, over $72,800 has been raised.

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Fundraising News

Please visit -

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.

Amazon Smiles for Kirby

Thanks to the shopping savvy of Kirby's cousin Molly, the Foundation is now registered with Amazon's Smile Foundation and is receiving .05% of the purchase price from her eligible AmazonSmile purchases. Just in time for holiday shopping! Are you an Amazon shopper? Then please consider logging onto smile.amazon.com and select The Children's Medical Research Foundation as the benefactor of your purchases. Already registered with Amazon? No worries, you can use the same account. The only difference is the smile on Kirby's face!

The Foundation Gives Thanks . . .

To **Mary and Christopher Sorensen** for choosing to honor Kirby and the Foundation on their wedding day with a donation in lieu of wedding favors;

To **Eileen Meyer**, Kirby's pre-school years speech teacher, for remembering Kirby with her donation;

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

To **Joanne Wilson**, Kirby's grandmother, for her donation in memory of **Joseph Zimmerman**; and

To **Barbara Cummings** for her donation in fond memory of her brother, **"Dicker,"** and **Curt Parker**.

Ashleigh Kidwell

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

To **Marlene and Bruce VanWagner** for their donation and kind words in honor of **Ashleigh and Brooke Kidwell**;

To **Judy Bible** and **Roberta and Arthur Kidwell** for their donations in celebration of **Ashleigh and Brooke's** birthdays; and

To the girls' parents, **Anna and Dave Kidwell**, for their support of the Sweetheart Dinner Dance and its auction with their annual road trip to Chicago, packed with items gathered throughout the year.

Brooke Kidwell

To **John Perrotto** and **Ken Healy** of **PMF Industries Inc.** for their most generous support of the Foundation in honor of **Hunter and Sydney Moff**;

Hunter celebrating his 22nd birthday with his dad.

To **Mr. and Mrs. John Houser** and **Noelle Nicholson** in celebration of **Hunter's** graduation; and

To **Betsy and Steve Fowler** for their donation in fond memory of their sweet daughter **Kimberly**.

(Continued on Page 4)

Fundraising News

Connecticut continues . . .

A note from Cynthia Logan

Rhianna celebrated her 23rd birthday with some very special friends. Farmer Buck from Hillside Equestrian Meadows brought two mini horses, Rocket and Little Johnny, and a "very friendly goat" Square Dot! Rhianna woke from a sound sleep at the sound of the horses and didn't stop smiling the entire hour of their visit! Square Dot took an immediate liking to Rhianna and was quite comfortable sitting in her lap. Farmer Buck signed a copy of his book "Cupid's Secret" to Rhianna. You can go to www.cupidandarrow.com to learn more about this true story of hope and miracles.

Rhianna's strength and perseverance are an inspiration to all. It is clear that her hope for a cure and her will to live have not been affected by her many challenges. Words cannot express the gratitude for the priceless gift of each smile from Rhianna. We are forever grateful to those who continue to support our fundraising goals and to the Wilson's for their steadfast commitment!

Supporters of **Rhianna Logan** continue their funding of **Rhianna's Hope** with donations from **Dennis Colgan, Laurel Daggett, Magdalena and Robert Logan, Donna and Alan Theriault, Donna and Cory Gabel, Donna Bernier and Martha and Jim Couture** and **Elaine and Roger Karabin**, as well as the many holiday donations received in Rhianna's name.

The **Manafort Family** again honored Rhianna by choosing the Foundation as the benefactor of its annual golf tournament. A $2,500 check was received "fore" Rhianna.

The Logans' **Purple Lemonade Stand** team, created last year to honor Rhianna's 22nd birthday, has raised $3,060 to date. The Logans' hope was that it would help to "step up their fundraising efforts." We'd say their stand is a great step up and testament to their community's enduring support.

Rhianna with her friend Square Dot.

Research Update

Dr. Kevin Flanigan
Nationwide Children's Hospital
Columbus, OH

Our team at Nationwide Children's Hospital has continued progress toward initiating clinical trials of gene therapy in MPSIIIA and MPSIIIB. The team has completed 12-month visits for the 25 subjects enrolled in the MPSIII Natural History Study. This study, which was supported in part by The Children's Medical Research Foundation, was designed to evaluate potential outcome measures for therapeutic trials, and to assess how these measures change in individual patients over time. Preliminary results have been presented at meetings of the American College of Medical Genetics in Salt Lake City in March 2015 and the American Society of Human Genetics in October 2015. The NCH team is currently preparing a manuscript for publication that will present the results to the MPS worldwide research community, and we will present results of the study at the WORLD symposium in San Diego in March 2016.

Critical data from this study were also included in an Investigational New Drug (IND) application that we have submitted to the FDA, requesting approval to perform a clinical trial of gene transfer for MPSIIIB. While we are still awaiting the formal response from the FDA, we are proceeding with the preparation of an IND submission for the MPSIIIA clinical trial, as well. Both gene transfer trials await completion of production and final release of the necessary viral vector.

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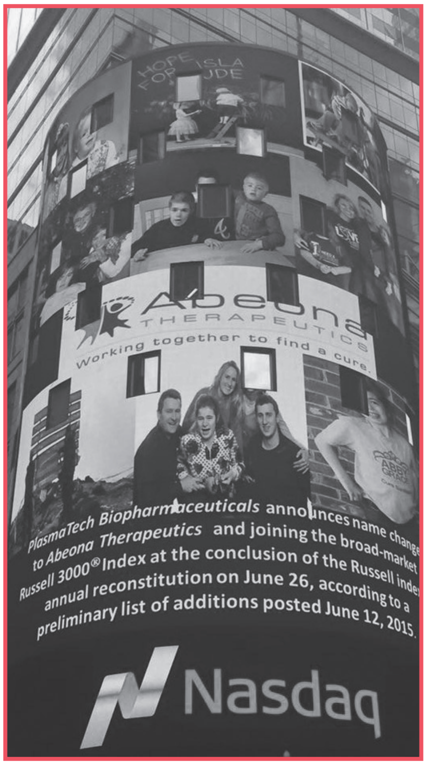
Research Update

Abeona Therapeutics

Abeona Therapeutics is focused on bringing forth gene therapy and plasma based products for severe and life threatening rare diseases. The company was created in 2013 through a close collaboration with a dozen Sanfilippo foundations to progress the work of Drs. Douglas McCarty and Haiyan Fu from Nationwide Children's Hospital in Columbus, Ohio. The collective efforts raised nearly $5 million dollars by the end of 2014 to start drug manufacture and steps to clinical trial.

In May of this year, the company was acquired by PlasmaTech Biopharmaceuticals, a publicly traded organization that had also been focused on treating rare diseases by utilizing a proprietary purification technology for selecting therapeutic proteins from human blood plasma. The newly formed entity chose to return to the Abeona Therapeutics name as it better reflected the commitment to rare or underserved diseases.

Abeona will soon begin the two gene therapy clinical trials for MPS IIIA and MPS IIIB, expected to first enroll in the US at Nationwide Children's Hospital late this year or early in 2016. We are very encouraged by the results we have witnessed pre-clinically with regard to the distribution to different areas of the brain, ability to address the systemic manifestations, and reduction of GAG's. This is to be a one-time dose, delivered by IV and has the ability to cross the blood brain barrier; a lower risk for complication compared to intrathecal or intracranial delivery. Additionally, we have recently added pre-clinical programs to focus to Fanconi's Anemia and different forms of Batten disease.



The Wilsons hit Times Square.
(upper left two pictures)

In Memory Of . . .

Warren Dendekker

Landon Stack with his mom, Ashley Riggs

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@comcast.net
to help us start a new, greener database of our supporters.

Our New Event

Denim and Diamonds

Friday, February 12, 2016

at the

Four Seasons Hotel Chicago

An elegant setting for an evening of fun

Join us for a casual evening with a buffet of fun food done the Four Seasons way accompanied by tasty bourbon cocktails, craft beers and a selection of wines.

Experience the electrifying DJ talent of KatV, who entertains with dynamic sets, fusing a blend of favorites with the best new indie artist works. She will elevate the evening with her electric violin performance. A feast for your senses!

And silent auction packages promise to tempt sports, travel, dining, spa and shopping enthusiasts.

Reservations are $125 per person 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

We have designated five sponsorship levels for the event, 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@comcast.net with any questions.

Denim and Diamonds

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, call (708) 784-0631 or visit us online at www.curekirby.org.**

Please include your phone number ____________________

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KirbyGram

Save the Date

Friday, February 12, 2016
for

Denim and Diamonds

For reservations visit us at

www.curekirby.org

or call (708) 784-0631

Join the fun February 12th for a casual evening with a buffet of fun food done the Four Seasons way accompanied by tasty bourbon cocktails, craft beers, a selection of wines, auctions, music and dancing.