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 2016

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

Dear Friends,

Last year I spoke of the fond memories we are so blessed to have with Kirby and continue to gather with each passing day. Realizing the importance of living in the moment and finding joy are what Kirby taught us long ago. "Happy?" is a question she asked daily - the memory of this question and her beautiful voice will never fade. We are grateful for our time with her and our bountiful memories. Kirby's unbounded resilience is inspiring - her comfort, our blessing.

2016 was an exciting time for progress in research. Nationwide Children's Hospital in Columbus, OH, along with Abeona Therapeutics Inc. in Cleveland announced that three children were enrolled and treated at Nationwide as part of the Phase 1/2 human trial for Sanfilippo type A. Just recently, they received approval for dose escalation in a second cohort for this trial. Plans for the Sanfilippo type B trial continue to progress, with hopes of a trial in the near future.

All of this excitement is tempered by the loss of precious children during the year and the heartache of their families. The passing of Rhianna Logan of Plainville, CT, and Elisa Linton of Toronto, Canada, particularly saddened Brad and me. Their parents, Cynthia and Gene Logan and Elisabeth and Randall Linton, were the first families to join in our efforts to find a cure 20 years ago. Our hopes for our girls were the same, and our friendship grew from our determination to find a cure for them.

Being mindful of these children and their families, Brad and I feel we must continue not only to honor lives lost but also to support the children whose lives depend on the success of these trials – eliminating this devastating disease – a cure for Sanfilippo Syndrome.

As we take what we hope to be our final turn toward the cure, we recognize it is all of you who have enabled us to be a part of this from its start. You have been champions of the cure for over 22 years, and it is your sustaining support of our mission that will lead us to the finish.

Wishing you happiness and joy this holiday season and beyond.

— Sue and Brad Wilson

Fundraising News

Denim and Diamonds

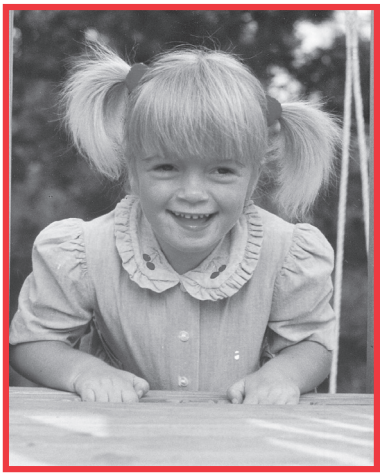
This new event for the Foundation was held February 12th at the Four Seasons Hotel in Chicago. Jamie Breslin, Director of Catering for the hotel who assisted Sue over the years with the Sweetheart, went above and beyond to create a beautiful setting and ensure that the new theme was carried out in every aspect of the evening. Over 100 guests were treated to the unique sounds of electric violinist Kat V, buffets of fun food done the Four Seasons' way, along with craft beers, bourbon cocktails and a selection of wines. The event raised over $55,000.

Sue Wilson comments, "Brad and I are grateful that our guests, many of whom have been to all 21 Sweetheart Dinner Dances, understand our changing times and our desire to see the decades of research through to a successful treatment for children worldwide."She continues, "It is these same people who were there for us during our most devastating time, as parents hearing Kirby's diagnosis,to lift our spirits and give us hope with their compassion and enduring support. We are honored by the depth of their devotion."

Kirby's Community

The list is seemingly endless when it comes to organizations and merchants within and around the Wilsons' hometown of Western Springs that have rallied around Kirby, her family and the Foundation's mission of a cure. In 1995, this community learned of what one news outlet described as a "little four-year-old, blue-eyed bundle of joy with an infectious smile" that had a rare and devastating disorder with no cure. And, she was the girl next door.

When Sue Wilson thinks of the impact this group has made with the grants the Foundation has awarded because of their support, as well as the kindness shown to Kirby, there is one word she thinks of first – "powerful." She explains, "From our first days to now, this community of believers has stood by our sides, doing whatever necessary to help in whatever way they could. The warmth we feel from their kindness and the impact their generosity has had on the progress of this Foundation and its mission create a story I hope makes people happy – something most important to our dear daughter that we know would bring a smile to her face." The Wilsons thank each and every individual, group and business for their unwavering support.



Kirby, 1995

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. Use this link http://smile.amazon.com/ch/36-4033667 for your direct access to benefit the Foundation. The only difference is the smile on Kirby's face!

The Foundation Gives Thanks ...

To **Debra and Robert Howard** for their donation in honor of Kirby and to **Denbury Resources, Inc.** for matching their gift;

To **Ameriprise Financial** for its generous donation;

To **Donna Logan-Gabel, Margaret Dawe, Raudel Gonzalez, Nicholas Megofna**, and that **secret someone** from **Macy's East** who designated the Foundation as their charity of choice in their employers' United Way campaigns;

To **Kirschbaum's Bakery** and **Casey's Market**, both from Kirby's hometown of Western Springs, for their continuous fundraising efforts

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Fundraising News

using cash jars. And to the people of the community who fill them and have helped to raise more than $541 to date this year, your "hometown girl" thanks you from the bottom of her little heart;

To **Daniel McPherson** for his donation to the Foundation in memory of **Elizabeth McPherson;**

To **Kathleen and Gerald Hofmann** and **Jay Pine** for their donations in memory of **Barbara Strand**; and

To **Barbara Cummings** for her most generous donation in memory of **Randy Bolduc**. Randy was a longtime friend of Sue Wilson who, along with Barbara and others, helped to form the Foundation and served as an Honorary Board member from its inception.

Finally, a special thank you to **Michael Lerich** for his donation and 21 years of entertaining guests with his wonderful musicians at the Foundation's Sweetheart Dinner Dances.

And, To The Families Working Together Toward a Cure . . .

We thank **Riverside Adventure Company, James Crouse** and **Drs. Margaret Crabtree** and **Glenn Bioiso** for their support in honor of **Hunter and Sydney Moff**.

Hunter and Sydney Moff

Angela and Luis Guajardo honored their daughter **Karina's 24th birthday** this past January with a donation to the Foundation toward its mission of a cure. Angela spoke of how cherished their memories are of Karina in her younger years.

Natey Coffey-Slattery

Olivia Poiselli of **Maine Laser Clinic** sent a donation in memory of **Nathanael Coffey-Slattery**. And his parents, **Rob and Wendy**, passed on a donation in memory of **"Natey"** from their mail carrier, **Brenda Demers**. In her note, Wendy described how touched they were to receive this donation along with a lovely card. Wendy went on to describe how Brenda had known Natey his whole life and what an important part of their daily lives this woman plays in their home in rural Maine.

Brooke and Ashleigh Kidwell were honored with donations from family friends **Marlene and Bruce VanWagner**, as well as grandparents **Roberta and Arthur Kidwell** and aunt **Judy Bible**.

Brooke Kidwell

And, once again, Brooke and Ashleigh's parents, **Dave and Anna**, loaded up their car with auction items for their annual trip to support this year's Denim and Diamonds event. And for years, the only room left in the car has been for devoted friends **Mary Ellen and Joe Bianco**, who travel with the Kidwells from Kentucky to support the Foundation's mission.

Ashleigh Kidwell

News From Connecticut

When Rhianna passed last December the Foundation received almost $5,000 in donations in her memory by the community of people that gathered around the Logans in support of "Rhianna's Hope" more than 20 years ago.

In addition to these donations, the **Artisan's Marketplace** continued with its annual **Stars for Hope** fundraiser and cash donation jar, which raised over $1,300. **Joanne Alfieri** donated to the Foundation, and her employer, LEGO, matched her contribution, doubling the donation.

The **Manafort Family** honored the memory of Rhianna by continuing to choose the Foundation as the benefactor of its annual golf tournament. A $2,500 check was received in memory of sweet Rhianna.

Also, Cynthia organized a raffle for two "incredible" quilts that her college roommate Caroline Murray donated, which raised $1,620.

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The Logan Family

A Note From Cynthia Logan

It is with unbearable sadness that I put pen to paper to share my thoughts. On Monday December 7th, 2015, our beautiful daughter Rhianna left this earthly world. For 20 plus years it was expected but yet feels so unexpected. The tremendous hope I had disappeared that morning as I held her, sitting on her bed as we had done for so many hours, days and years. Rhianna's incredible strength, grace and precious smile never waned, not even in her final days.

Only a few weeks later came incredible news that there was a child being treated for Sanfilippo. A beautiful little girl, the same girl that appeared on my computer screen one night not long ago, no mention of Sanfilippo, but I knew when I clicked on the story it would say Sanfilippo. The newscast of her parents was almost word for word what Gene and I had said and felt when Rhianna was diagnosed so long ago in 1995. As the story ended with the child running, she looked just like Rhianna at that age.

My dream of Rhianna being the first child treated is over, so the peace in knowing that her journey has changed the lives of others is a great comfort. It was Rhianna's intense determination, her incredible smile and her love of life that moved so many people to take action and make a difference, creating awareness and funding research. Our blessings go out to all the families working so hard to fund a cure and to the children in trials.

We will forever be grateful to each and every person who supported Rhianna's Hope over the many years, and our hearts are consoled by those who continue to do so. There are no words grand enough to thank Sue and Brad as they continue to work to fund the trials and the ongoing research while they care for dear Kirby. Our hope remains strong for all the children who await a cure.

A Young Rhianna

Rhianna and I had a favorite bedtime book called ON THE NIGHT YOU WERE BORN by Nancy Tillman. I close with a quote from that book in honor of Rhianna: "for never before in story or rhyme (not even once upon a time) has the world ever known a you, my friend, and it never will, not ever again…heaven blew every trumpet and played every horn on the marvelous, wonderful night you were born."

Elisabeth Linton Shares Her Words to Elisa

Randall, Elisa and Elisabeth Linton

Below are some of the words Elisabeth Linton wrote to her dear daughter Elisa after her passing in November.

My Dearest Elisa, it was a privilege, joy and blessing to be your mom.

You have given me a whole new view of the meaning of life and taught me to see the joy and beauty in the simplest of things, never to take anything for granted, and the true meaning of the words "unconditional love," something you gave me daily. You loved and had a zest for life like no one else I've ever met. God never promised us that everything in our life would be good, but he did promise that all would work together for good. I am forever grateful to God for the 22 and a half years I cared for and loved you and it won't stop.

Elisa, I was so proud of the way you lived. When life gave you a hundred reasons to cry and give up, you didn't. You had a joyful spirit and innocence in the midst of pain and limitations. You fought for every milestone in your life and there were many. You were strong, brave, and a courageous fighter who laughed and smiled your way through life.

Lissy, you never had a boyfriend or went out on dates, but you were loved by thousands of people and received hundreds of red roses in your short life.

You never attended university, but you enabled research at universities and hospitals around the world to advance medical science in a significant way.

You never drove a car but drove change in the world of rare genetic disorders, affecting thousands of children worldwide.

You never spoke a word past your 12th year, but you spoke on behalf of Sanfilippo children and their families around the globe, silently spreading the word about this horrible disease and the urgency of finding a cure.

Your life wasn't one with huge successes but one of significance.

We prayed a cure would come in your lifetime, and I know we are on the road to one. But God chose to heal you in a different way. You lived to see clinical

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Words to Elisa continued

trials start, ones that your life helped to inspire, and to be a bridesmaid in your sister's wedding six weeks ago. You're now in a better place, dancing, running, laughing, singing, eating, hugging and even pinching. Yes, I will even miss your pinches. You were great at that.

People say you're never the same after a loved one leaves you, but don't worry about me, I don't want to be the same. You left an imprint on me that will never be removed. I want to be a better person because I knew and loved you. Our world is different now without you. It's a better place. I shed tears because you are gone and not in my arms, but rejoice because you lived with love and purpose and are now in Jesus' arms. And I am trusting God to help us through this difficult time ahead without you. In God I have comfort, in God I have strength and in God I have hope. And we are surrounded by amazing friends from within the beautiful community you have created.

I feared for the day when I would have to say goodbye. And now that it's here, I'm counting on drawing on your strength to deal with it. I will live each new day embracing the joy and love you brought to our family and countless others and the impact you had in this world.

Good-bye my sweet princess…until we meet again. Love you forever, love you for always, as long as I'm living my baby you will be.

Elisa, the Linton's "shooting star"

Research Update

Abeona Therapeutics Inc., New York, NY, and Cleveland, OH, issued press releases on October 20th and 25th, 2016.

Excerpts from these releases follow:

October 20, 2016, Abeona Therapeutics Inc. (NASDAQ: ABEO), a clinical-stage biopharmaceutical company focused on developing therapies for life-threatening rare genetic diseases, provided today at the Orphan Drugs & Rare Disease Conference (London, UK), an update on clinical results through 30 days post-injection for the completed low-dose cohort (n=3) in the ongoing Phase 1/2 trial for ABO-102 (AAV-SGSH). The first-in-man clinical trial utilizes a single intravenous injection of AAV gene therapy for subjects with MPS IIIA (Sanfilippo syndrome type A), a rare autosomal recessive disease affecting every cell and organ in the body causing neurocognitive decline, speech loss, loss of mobility, and premature death in children.

The ongoing Phase 1/2 study is designed to evaluate safety and preliminary indications of efficacy of ABO-102 in subjects suffering from MPS IIIA. Observations 30 days post-injection for the low dose cohort demonstrated:

- ABO-102 is well-tolerated in subjects injected with the low dose of 5E13 vp/kg ABO-102 with no treatment related adverse events or serious adverse events (SAEs). Following favorable review of the safety data by the independent Data Safety Monitoring Board (DSMB), enrollment in the high dose cohort has commenced.

- In the natural history study evaluating MPS III subjects it was shown that urine and cerebral spinal fluid GAG (heparan sulfate or "HS") are significantly elevated in the subject population as a symptom of disease pathology.
- All subjects in the low-dose cohort experienced reductions from baseline in both urinary HS and CSF. At 30 days post-injection, urinary HS reduction was 57.6% +/- 8.2%. Reduction in CSF HS was 25.6% +/- 0.8%, suggesting that ABO-102 crossed the blood brain barrier after intravenous administration.
- The natural history study in 25 subjects with MPS III (Truxal et. al., 2016, Mol. Genet. Metab.) demonstrated that subjects had increased liver and spleen volumes averaging 116% and 88%, respectively, at baseline that did not change over a year of follow up.
- All three subjects demonstrated significant reductions in liver volume (17.1% +/- 1.9%), and spleen volume (17.6% +/- 7.1%) from baseline, as measured by MRI at 30 days post-injection.

Per the design of the clinical trial, subjects in the low-dose cohort received a single, intravenous injection of AB0-102 to deliver the AAV viral vector systematically throughout the body to introduce a corrective copy of the gene that underlies the cause of the MPS IIIA disease. Subjects were evaluated at multiple time points over the initial 30 days post-injection for safety assessments and initial signals of biopotency, which suggest that ABO-102 successfully reached target tissues throughout the body, including the central nervous system, to reduce GAG content that underlies the lysosomal storage pathology central to Sanfilippo syndrome type A (MPSIIIA).

"We remain encouraged by continued signs of tolerability and by early

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signals demonstrating reduced urinary and CSF GAG," stated Kevin M. Flanigan, MD, principal investigator with the Center for Gene Therapy at Nationwide Children's Hospital and Professor of Pediatrics and Neurology at The Ohio State University College of Medicine. "Additionally, we are informed by the natural history study that subjects with MPS IIIA experience hepatosplenomegaly, and are pleased by observations in the low dose cohort of significant reductions in liver and spleen volumes 30 days post-injection as measured by MRI."

A more complete analysis of these data will be presented from the low-dose cohort and initial high dose cohort at a scientific conference in the first quarter of 2017. The Data Safety Monitoring Board has approved dose escalation of the high dose cohort this quarter.

"The data demonstrate an early and robust systemic delivery of ABO-102, and the reductions in CNS and urinary GAG support our approach for intravenous delivery of ABO-102 for subjects with Sanfilippo syndromes," stated Timothy J. Miller, Ph.D, President and CEO of Abeona Therapeutics. "We are excited about early biomarker signals in this trial, including the reductions in liver and spleen volumes. Positive impact on hepatosplenomegaly has been an important measure historically in other clinical programs in the lysosomal storage disease space."

Abeona's MPS IIIA program, ABO-102, has been granted Orphan Product Designation in the USA and received the Rare Pediatric Disease Designation, and recently announced Orphan Drug Designation has been granted in the European Union.

October 25, 2016, Abeona Therapeutics Inc. (NASDAQ: ABEO), a clinical-stage biopharmaceutical company focused on developing therapies for life-threatening rare genetic diseases, announced today that the U.S. Food and Drug Administration (FDA) granted Fast Track designation for ABO-102, a single intravenous injection of AAV gene therapy for subjects with MPS IIIA (Sanfilippo syndrome type A), a rare autosomal recessive disease affecting every cell and organ in the body causing neurocognitive decline, speech loss, loss of mobility, and premature death in children.

"Fast Track designation underscores the importance that the FDA places on developing new treatments for life-threatening disorders, such as MPS IIIA, and reinforces our mandate of accelerating the development of ABO-102 to market," stated Timothy J. Miller, Ph.D., President and CEO of Abeona Therapeutics. "We look forward to providing additional updates for our ongoing Phase 1/2 clinical trial."

Fast Track designation is a process designed to facilitate the development and expedite the review of drugs to treat serious conditions that address an unmet medical need. Advantages of Fast Track designation include opportunities for more frequent interactions with the FDA during all aspects of development, and eligibility for priority review and accelerated approval. This designation is in addition to ABO-102 being granted Orphan Drug designation by the FDA and the European Medicines Agency (EMA), as well as having already received the Rare Pediatric Disease Designation from the FDA.

"The Fast Track designation comes with an increase in interaction and feedback from the FDA during the development process of a drug and signifies that the FDA may be able to expedite the review and approval of the ABO-102 gene therapy product which, in preclinical and initial clinical work, has shown encouraging signals of biopotency," stated Steven H. Rouhandeh, Executive Chairman. "This designation also demonstrates to the children and families afflicted with MPS IIIA, the FDA's recognition of the severity and importance of addressing this rare orphan disease."

Michelle Berg, Vice President Patient Advocacy for Abeona speaks of the releases' highlights:

October 20, 2016 "What is notable is the continued tolerance, or safety, demonstrated so far, as this is the primary focus for this study. Additionally, the sizes of the liver and spleen have been reduced in all three children, as well as reduced quantities of the glycosaminoglycans (specifically heparan sulfate), or built up long chain complex sugars, in both the urine and the spinal fluid. What this shows is that within 30 days after the single intravenous injection there is evidence that the AAV carrying the corrective gene is crossing the blood brain barrier and having a biological effect. This is still early but we are encouraged by this information and look forward to collecting more information and sharing with the community.

October 25, 2016 "Fast Track designation is an important achievement that complements our approach to reach approval safely and swiftly for as great of impact to as many children as possible. Here are a handful of reasons why this designation will be helpful:

- More frequent meetings with FDA to discuss the drug's development plan and ensure collection of appropriate data needed to support drug approval
- More frequent written communication from FDA about such things as the design of the proposed clinical trials and use of biomarkers
- Eligibility for *Accelerated Approval and Priority Review, if relevant criteria are met*
- *Rolling Review*, which means that a drug company can submit completed sections of its Biologic License Application (BLA) or New Drug Application (NDA) for review by FDA, rather than waiting until every section of the NDA is completed before the entire application can be reviewed. BLA or NDA review usually does not begin until the drug company has submitted the entire application to the FDA"

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.

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.

A New Way to Celebrate

February, 2017

A new evening of celebration and fun will be at your place.

Events over this past year have reminded Brad and me of the importance of family and never taking for granted or missing the opportunity to hold them close. Taking the time for holding hands and happiness is something Kirby would insist upon daily while singing "We're a happy family, with a great big hug and a kiss from me to you, won't you say you love me too!" Little did we know how close to our hearts we would hold these now precious moments in our lives.

With this in mind, we have decided not to plan an event for this February. Instead, Brad and I will pick a night in February to have some family fun, to think of you and celebrate the love and support you have shared with us, all while holding Kirby tight. We ask that you, too, gather your family and enjoy whatever makes you happy together. Celebrate your family. Just don't forget the hugs and kisses for Kirby.

Be sure to let us know what fun you had with your family. We'd love to see your pictures and share your happy stories.

The work of the Foundation continues

The work of the Foundation continues, as we have issued more than $222,000 in funding toward the launch of Sanfilippo type B clinical trials beginning soon. Our help will be needed again. At this important turning point, we hope that you will consider the Foundation in your year-end charitable donations.

Donations can be made on line at www.curekirby.org/events or by mail to: The Children's Medical Research Foundation, P. O. Box 70, Western Springs, IL 60558.

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