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 2017

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

Dear Friends,

Events of this past year have brought clarity to Brad's and my direction for the Foundation that energizes our resolve and gives us a sense of fulfillment in our mission. Events have also solidified our thoughts on spending time with Kirby.

I am speaking of both the excitement we feel toward research and the emotions that evolved from the heartache Brad and I felt from the loss of some very precious children in recent years. These emotions have made it clear that the time had come for me to devote my time to be by Kirby's side.

Spending my days with Kirby is time I do not take for granted. Being able to care for her in the manner she so richly deserves is my honor and blessing. I look into her eyes and reflect on her courageous journey that has enabled research and has empowered so many around her to put their adversities and frustrations in perspective with her one simple question, "happy?" Her smile and spirited determination for happiness are and will forever be our fondest and most powerful memories.

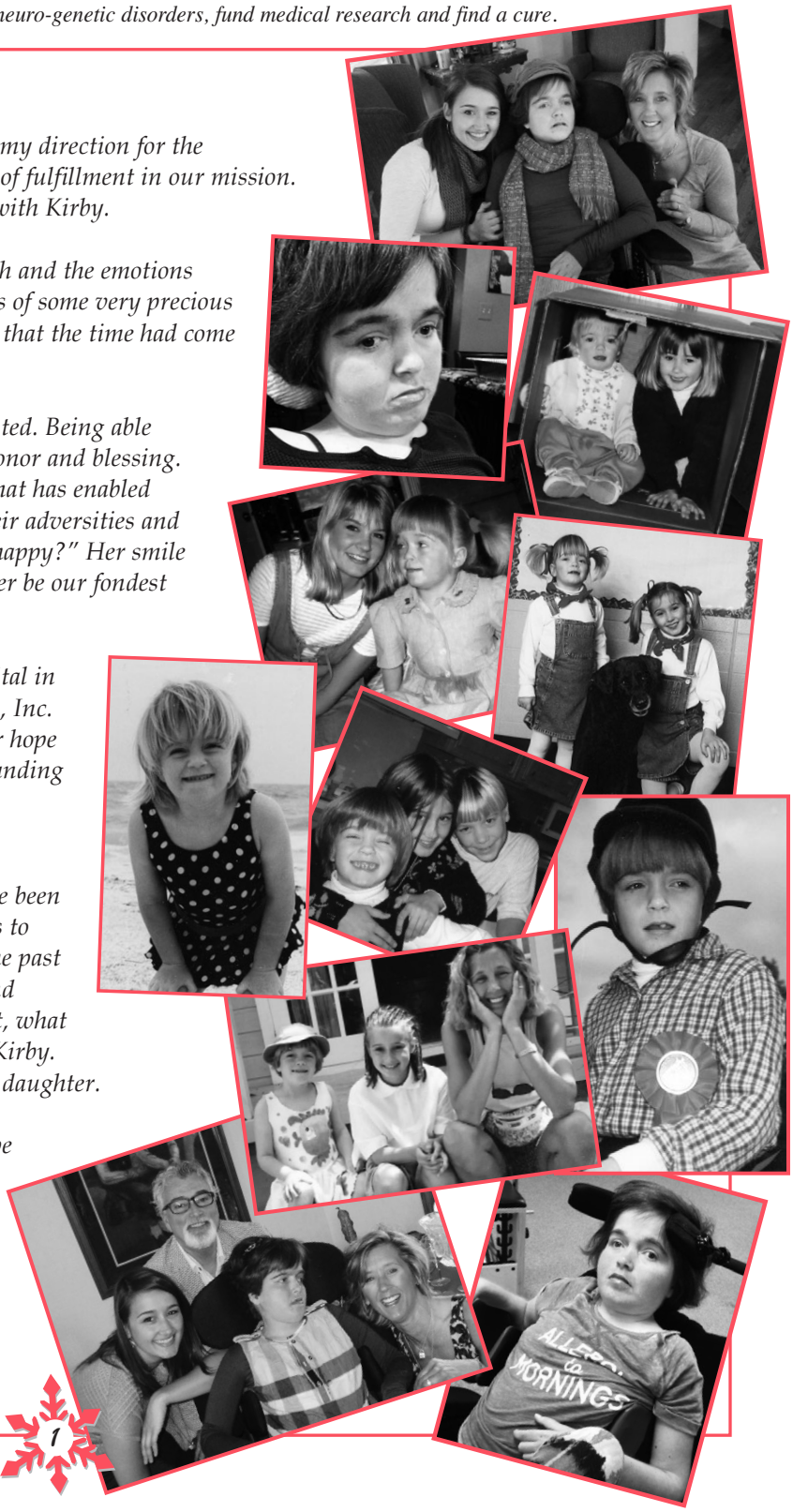
The trials being conducted at Nationwide Children's Hospital in Columbus, OH, in collaboration with Abeona Therapeutics, Inc. in Cleveland continue to progress with results that fuel our hope for a cure. Our most recent opportunity to be a part of expanding these trials and allowing treatment for more children with Sanfilippo is everything we want for the Foundation.

These are exciting times for children worldwide, which have been made possible by many. But to us, our heartfelt thanks goes to you, whose compassion for our daughter and family over the past 22 years will never be forgotten. A community of family and friends that gathered and created a formidable front against, what we were told, was an incurable disease became A Cure for Kirby. What a remarkable story that will eternally honor our dear daughter.

As my days with Kirby fulfill my life as her mother, we hope you will continue to support the Foundation's most important mission of a cure for all children afflicted.

With gratitude and joy-filled wishes,

—Sue and Brad Wilson



Fundraising News

Kirby's Community

The list is seemingly endless when it comes to organizations, merchants, churches and school groups 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" who 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 throughout the years, she is deeply moved. "From our first days to now," Sue explains, "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 generosity and the effect it 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.

Amazon Smiles for Kirby

Are you an Amazon shopper? Then don't forget that the Foundation is registered with Amazon's Smile Foundation and receives .05% of the purchase price from your eligible Amazon purchases. With holiday shopping fast approaching, your contributions can really add up for the Foundation. 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 **Donna Logan-Gabel, Margaret Dawe, Nicholas Megofna, Mark Leavitt**, and that **secret someone** from Hartford, CT, who designated the Foundation as their charity of choice in their employers' United Way campaigns;

To **The James Jay Lavoie Memorial Fund** for its donation to the Foundation;

To **Rob Credit** for his and **Alison's** donation that was matched by Rob's employer, **Medtronic**;

To **Paul Prinke** for choosing the Foundation as the recipient of **The VP Boys Club Making A Difference Charity's** annual donation;

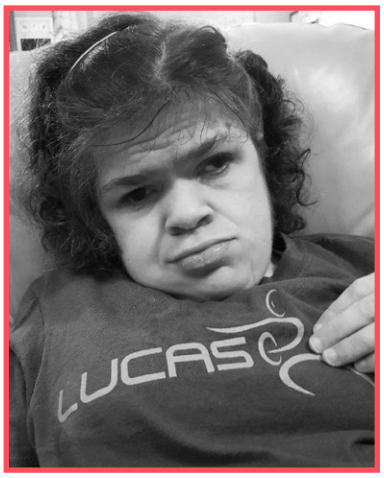
To **Rita and Sam Girgis** for their donations each year honoring Kirby;

To **Barbara Cummings** for her most generous contribution in celebration of family, and

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 $665 to date this year, your "hometown girl" thanks you from the bottom of her little heart;

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

Brooke Kidwell



Ashleigh Kidwell

To **"Grandma and Grandpa Kidwell"** for their donation honoring **Brooke and Ashleigh Kidwell's** birthdays and to **Mr. and Mrs. Jimmy Bowen's contribution** in the girls' honor;

To **Diane Peeling** for her donation honoring **Sydney and Hunter Moff** of Williamsport, PA, and

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

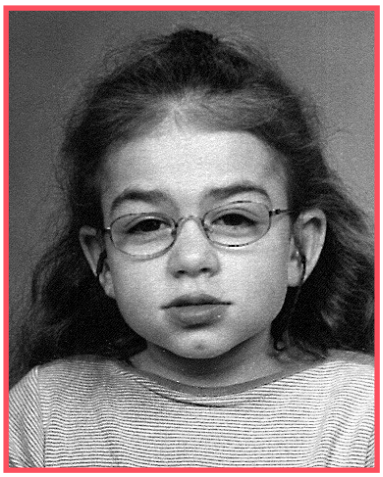
(Continued on Page 3)

Fundraising News continued

The Foundation would also like to give thanks to the many people who donated in memory of another child lost to this devastating disease. **Carley G. Trocheck,** of Ravenna, OH, was freed from the confines of

Carley Trocheck

Sanfilippo on January 28th at the age of 22 years. Her mother, **Barbara McFarlan Trocheck** reflects on her life, "Carley was a special little lady who inspired love in all she met. She loved her little brother Max so much she couldn't be very far from him –



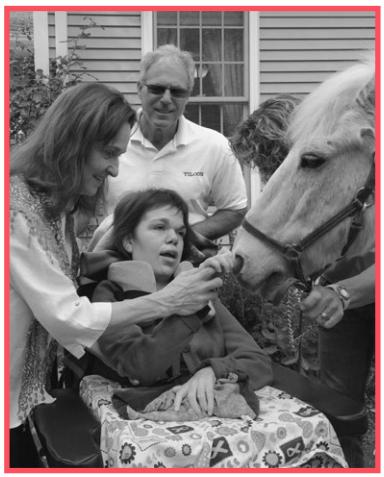
Carley Trocheck

EVER." Carley's love and goofy giggles will be fondly remembered by family and friends.

We thank Barbara for her generous gift memorializing both her mother and Carley.

News From Connecticut

The **Manafort Family** honored the memory of Rhianna by continuing to choose the Foundation as the benefactor of its annual golf



Rhianna Logan

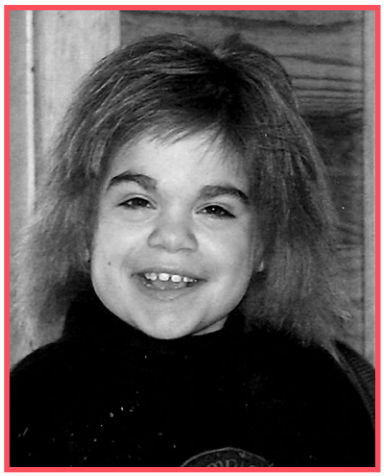
tournament. A $2,500 check was received in memory of sweet Rhianna.

The quilt raffle concluded with two lucky winners of these handmade treasures, which were created by Cynthia Logan's college roommate, Caroline Murray. The Foundation received $1,978 from ticket sales.

We thank the many family members and friends of Cynthia who donated in memory of her father, James Couture.

A Note From Cynthia Logan

When all you have is memories, you pray that you do not forget a single one. We are grateful for each memory and for all the truly wonderful time we had with Rhianna. December 7, 2017, marks two years since she lost her battle with Sanfilippo. Some days it feels like yesterday, other days it feels like forever. On October 22nd we honored Rhianna with a memorial service at the "Cherish Our Children Angel Statue" in Newington, CT. Rhianna would have celebrated her 25th birthday on October 15th. Our hearts were overwhelmed and full of gratitude as over 60 friends and family arrived. The weather was perfect, I am sure Rhianna had something to do with that, and the service was beautiful, thanks to all who participated. It was a testament to how amazing Rhianna always was and continues to be. The paver we placed in her honor reads "Amazing Soul." We thank everyone who continues to donate on Rhianna's behalf, and we are forever grateful to Sue and Brad Wilson, whose vision, diligence and years of dedication have driven research to where it is today. I still believe in a cure, I still have hope that it will be soon and I look forward to knowing MPS families who have been blessed by the success of medical research.



Rhianna Logan

Research Update

Let The Trial Begin!

Recently, Michelle Berg, Vice President, Patient Advocacy for **Abeona Therapeutics, Inc**. in Cleveland, OH, announced the listing of the Phase I/II Gene Transfer Clinical Trial for Sanfilippo Type B (Kirby's sub-type) on the clinicaltrials.gov website, where information is listed on the trial and enrollment criteria. Michelle stated, "This work has been years in the making, involving many from the various foundations around the world, researchers at Nationwide, and families who participated in the Natural History Study. We are thankful to all who have helped to make this next critical stage of the research and understanding of results a possibility. Further, we appreciate those who will eventually participate in this investigation as well as those who will watch with interest and continued encouragement."

Sue Wilson comments, "For us, this day has been 22 years in the making, and we are grateful the day has finally come for children like Kirby to have the opportunity of a future without Sanfilippo.

"Brad's and my resolve is further energized by our most recent contribution to these trials, which will ultimately help to expand the enrollment opportunities, allowing treatment for more children" Sue adds. This collaboration was announced by Abeona on October 16th and is detailed below in excerpts from Abeona's press release.

The Foundation is thrilled with the progress of the Sanfilippo Type A human trials that are being conducted at **Nationwide Children's Hospital** in Columbus, OH, in conjunction with Abeona. Excerpts from Abeona's press releases provide details along with its announcement of the groundbreaking for a commercial gene therapy manufacturing facility in Cleveland named The Elisa Linton Center for Rare Disease Therapies, honoring the memory of Elisa Linton of Toronto, Canada. It is wonderful tribute to her life and to the years of work by her family through their foundation, the Sanfilippo Children's Research Foundation.

Oct. 04, 2017 Abeona Therapeutics Inc. (Nasdaq:ABEO), a leading clinical-stage biopharmaceutical company focused on developing novel gene and cell therapies for life-threatening rare diseases, announced today the groundbreaking of the first anticipated commercial gene therapy manufacturing facility in Ohio. The Cleveland-based facility, named The Elisa Linton Center for Rare Disease Therapies, will have the capacity to produce advanced gene and cell therapies to treat serious and debilitating rare diseases. The dedication and groundbreaking ceremony is being held today, October 4, 2017.

"We are very excited to announce the creation of The Elisa Linton Center for Rare Disease Therapies, which will be a global resource for production of gene therapies with the potential to bring new treatments to rare disease patients around the world," said Timothy

J. Miller, Ph.D., President and CEO of Abeona Therapeutics. "It is especially fitting that this center is named for Elisa Linton, who was born with Sanfilippo syndrome, a rare terminal disease. The memory of Elisa and courage of her family continue to be a great inspiration to all members of the rare disease community."

The Elisa Linton Center for Rare Disease Therapies will initially be used to produce ABO-101 and ABO-102, investigational gene therapies currently in development at Abeona for the treatment of patients with Sanfilippo Syndrome, and EB-101, an investigational autologous

(L-R) Randall and Elisabeth Linton, Tim Miller, Jessica Linton Mason, Connor Linton and Michelle Berg

(Continued on Page 5)

cell therapy for the treatment of recessive dystrophic epidermolysis bullosa (RDEB), a rare and devastating skin disorder. The Center will also house Abeona's expanded viral-vector lab, which will develop and produce unique and proprietary vectors used for the delivery of gene therapies. The 6,000 square foot Center will be built out and validated over the next 12 months.

Oct. 11, 2017 Abeona Therapeutics Inc. (Nasdaq:ABEO), a leading clinical-stage biopharmaceutical company focused on developing novel gene therapies for life-threatening rare diseases, announced today that two patients were enrolled in the Company's ABO-102 Phase 1/2 clinical trial, at sites in Australia and the US. Both patients have been treated with the Company's Cohort 3 dose of ABO-102 (3 x 1013 vg/kg).

"The recently announced one-year data on our Cohort 1 patients showed durable, time-dependent responses as measured in reductions of heparan sulfate storage pathology in the CSF and urine, reduction in liver volume, stabilization of deep brain architecture and signals of stabilization of neurocognitive decline one-year post-administration. After seeing dose-dependent improvements in Cohort 2, with ABO-102 being well tolerated to date, Abeona, together with our principal investigators, dose-escalated to potentially enhance clinical benefits and prolong durability; a decision supported by the regulatory agencies across the three countries supporting our trial," stated Timothy J. Miller, Ph.D., President and CEO of Abeona Therapeutics. He continued, "ABO-102 continues to be well-tolerated at all doses at all follow-up timeframes, and has enabled an accelerated enrollment schedule over the coming months. We look forward to reporting additional clinical data in the ABO-102 global trial later this year."

Per the design of the pivotal expansion, subjects in the ABO-102 trial receive a single, intravenous injection of ABO-102 to systemically deliver the AAV viral vector throughout the body and CNS, introducing a corrective copy of the SGSH gene that underlies the cause of the MPS IIIA disease. Subjects are evaluated at multiple time points post-injection for safety assessments and initial signals of biopotency and clinical activity, which indicate that ABO-102 successfully reached target tissues throughout the body, including the central nervous system.

Oct. 16, 2017 Abeona Therapeutics Inc. (Nasdaq:ABEO), a leading clinical-stage biopharmaceutical company focused on developing novel gene and cell therapies for life-threatening rare diseases, announced today a collaborative agreement between nine Sanfilippo foundations to provide approximately $13.85 million of grants to Abeona in installments for the advancement of the Company's clinical stage gene therapies for Sanfilippo Syndrome Type A (MPS IIIA) and Sanfilippo Syndrome Type B (MPS IIIB).

"Abeona is pleased to continue our global collaboration with the Sanfilippo foundations to help further advance our gene therapy programs for MPS III disease," said Timothy J. Miller, Ph.D., president and chief executive officer of Abeona Therapeutics. "The effort and expertise that we continue to commit to the ABO-102 and ABO-101 programs puts us in a strong position to further extend the important progress reported to date. We are grateful to the foundations for their ongoing commitment to identifying and facilitating the development of clinical innovation to treat patients with MPS III disease."

"Stop Sanfilippo considers that, based on the very good clinical data recently published by Abeona on the Phase I/II trial, this is a great opportunity to support a further step on this program, making it possible to treat more patients and allowing a broader clinical indication for this potential gene therapy potential treatment," said Emilio Lopez Alvarez, President of Stop Sanfilippo in Spain.

Team Sanfilippo Foundation, Stop Sanfilippo Fundación, Fundación Sanfilippo B, Sanfilippo Children's Foundation, the National MPS Society, the Red Sanfilippo Foundation, The Children's Medical Research Foundation, Abby Grace Foundation, and Fondation Sanfilippo Suisse collectively collaborated on the grant to Abeona.

"The importance of reducing the heparan sulfate as a cause of disease burden cannot be understated, and the clinical data demonstrated by Abeona enabled us to provide additional support in the pursuit of finding new paradigms to treat all children with Sanfilippo Syndrome," stated Carl Kapes, Board Member of Team Sanfilippo.

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.

Look For News Coming Soon On Our 2018 Fundraising Event

The work of the Foundation continues

As you have read, the work of the Foundation continues, research progresses and opportunities continue to arise to expand and accelerate the efforts toward a cure. At this encouraging and critical juncture in our journey together, we hope that you will consider the Foundation in your year-end charitable donations and help us to fulfill our mission of the cure.

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

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