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 2019

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

Dear Friends,

Brad and I know, as all parents do, that the depth of love for our children has no boundaries. What we chose not to dwell on after Kirby's diagnosis, just before her fourth birthday, was the depth of our grief if we lost her. It was unimaginable, and there was so much that could be done to change the devastating outcome of Sanfilippo. The Foundation was formed, decades of fundraising, awareness, and in turn, expansion of research followed, including Sanfilippo in all the most promising methodologies of treatment. All of this, while watching Kirby capture the hearts of all those she met, rallying a community and inspiring determination with her unbounded personality filled with fortitude and grace and her ever-important question, "Happy?" Unfortunately, that unimaginable grief became a reality for Brad and me on October 22nd when our beloved Kirby passed away unexpectedly, yet peacefully, in her sleep. Our hearts are broken.

The outpouring of sympathy was and continues to be overwhelming and shows us that, like her fortitude and will for happiness, her impact on others has no boundaries and will continue to influence. She will forever be our family's inspiration and a beautiful face of hope.

We thank all of you for your sympathy, for sharing your memories and for expressing how Kirby's life radiates into yours. It is your stories and reflections, along with our memories, that will help us as we begin our new journey. And, with your continued support of this Foundation and its mission, you will help us to ensure Kirby's legacy – A Cure for Kirby.

With heartfelt gratitude,

—Sue and Brad Wilson

Fundraising News

Facebook and #GivingTuesday

For Kirby's sister, Maggie, there wasn't a second thought to using this opportunity to help. With her love for Kirby and her knack for all things digital, Maggie knew this fundraiser had success written all over it! Her many Facebook friends agreed and contributed more than $5,000 on last year's #GivingTuesday held November 27th.

The Foundation Gives Thanks . . .

To **Donna Logan-Gabel** and **Nicholas Megofna**, who designated the Foundation as their charity of choice in their employers' United Way campaigns;

To **Barbara Cummings** for her generous donations in memory of **Adele Orr Delisi** and **Judy Davis**;

To **Margaret Anderson** for her donation in honor of **Gail and Bill McCabe;**

To **Betty and Bruce Tompkin** for their generous support of research in honor of Kirby;

To **Jan and Don Matheny** for their donation in honor of **Donna and Matt Scotty**;

To **Beth and John Wilson**, Brad's brother and sister-in-law, for their donation through John's employer's **JPMorgan Chase's Good Works Workplace Giving Program**;

To our **secret Facebook Friends** for donating to our mission;

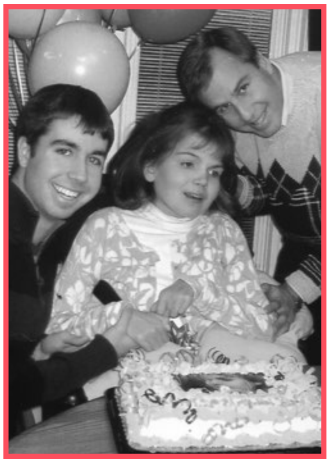
To the **Amazon shoppers** who designate the Foundation as their charity of choice on smile.amazon.com. Amazon's Smile Foundation has been donating .05% of your Amazon purchases to the Foundation. We are sure Kirby is smiling down on this!

To **Kirschbaum's Bakery** and **Casey's Market**, both from Kirby's hometown of Western Springs, for their continuous fundraising efforts using cash jars over the past 25 years. And to the people of the community who filled them, the Wilsons thank you for filling their hearts with the warmth of your compassion for their daughter, your "hometown girl," and

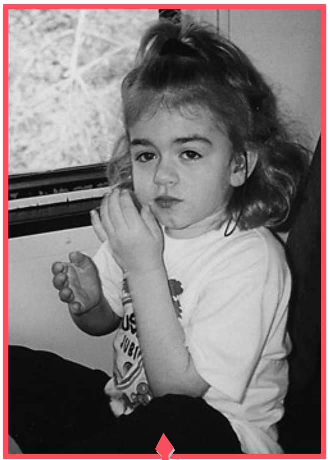
To all who donated to the Foundation in memory of Kirby, the Wilsons thank you for honoring their daughter's life by helping them to turn others' hope of a cure into a reality.

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

To **Betsy and Steve Fowler** for their kind words and donation in fond memory of their daughter, **Kimberly**;



To **Barb Trocheck** for her donation in loving memory of her daughter, **Carley**;



To the many that donated in fond memory of dear **Sydney Moff**;

To all who donated in memory of **Arthur Kidwell**, father of **Dave Kidwell**, and to the many family and friends who donated in memory of Anna and Dave's daughter **Ashleigh**;

To **Danielle and Matthew Cuculic** for their donations, as well as to their employer, the **Humana Foundation**, for its matching gift honoring Danielle's sisters, **Brooke and Ashleigh Kidwell**.

To the **Manafort Family**, who honor the memory of **Rhianna Logan** 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;

To the people who contribute to the **Artisan Shop's** cash jar, as well as family and friends who donate to the Foundation, Rhianna's parents, **Cynthia and Gene Logan**, give their heartfelt thanks for always remembering "Rhianna's Hope" and Cynthia's words, "Our work is not done."

Sydney Moff
May 6, 1989 – May 4, 2019

Theresa and Steve Moff of Williamsport, PA, lost their beautiful daughter Sydney, on May 4th at the age of 29.   Her family, which includes brothers Stefan and Hunter, reflects:

"We lost our bright ray of sunshine. Her smile and laughter brightened our world and will forever touch and brighten our hearts and souls. Despite her illness, she took life on with gusto. She was up for anything. Nothing was ever not worth experiencing. She also never complained about her illness or any of the painful and exhausting hardships she had to endure. We will forever remember her laughing, singing and dancing her way through life. Even though in the end, her illness robbed her of the ability to physically experience so many of her joys, you could still feel it in her bones and see it in her eyes, especially at "cuddle time."

Theresa remembers the words a dear friend spoke to her upon learning of Sydney's passing: "She told me not to think of everything that I had lost by her dying but everything I had gained by having her in our lives. Those words have so helped me focus on the good things, and there were many. It has brought many smiles to my face and brought back wonderful memories."

Ashleigh Kidwell
May 29, 1990 - January 2, 2019

Ashleigh Kidwell, daughter of Anna and Dave, sister to Danielle, Callie and Brooke, died on January 2nd at the age of 29. The Kidwells share Danielle's remembrance of their dear daughter and sister.

"Ashleigh had a hard life, and one that was very unfair. Looking back through pictures, I realized and was reminded of how much Sanfilippo has taken from her- her abilities, her independence, her personality- slowly, over her almost 30 years. But going through pictures also reminded me that Ashleigh had a good life. There has been plenty of laughter, activity, and fun. We went camping, to the zoo, to the beach, hiking. School field trips, gymnastics classes. Family mountain camping trips to Colorado and Utah. She was a sister, daughter, girl scout, school student, gymnastics student. She loved music, musicals, and to sing. She was a daddy's-girl.

Ashleigh — who we also called Ash, Nellie, or Nel — also gave us many things as a family. Even after Sanfilippo had taken much from her, caring for her kept us closer as a family. We learned teamwork. We ate the majority of our dinners together as a family. We spent more time together as a full family of six. We divide and conquer. We have definitely learned that sometimes when things get too crazy or ridiculous, all you can do is laugh. We learned all the words to Mary Kate and Ashley movies, to enjoy hearing Brooks and Dunn's Boot Scootin' Boogie and Tim McGraw's I Like It, I Love It over and over again. For a while there we learned to strategically place ourselves between Ashleigh and any fire alarms, because for whatever reason she had to pull them, like a moth to a flame. She was on a first name basis with the fire department for her school!

Ashleigh will continue to be here with us through her impact on those who knew her. She changed all of us, and I'd say made all of us better. Having special needs sisters makes me more compassionate. More accepting. More understanding. Convinced me that the most important thing I can teach to my future children is to be inclusive. My parents initially got our dog Zoe for Ashleigh. Turns out she seemed indifferent, but Zoe will forever be my favorite dog, so thanks Ash for that! The first Christmas when my husband Matt and I were dating, he wrote cards to Ashleigh and Brooke, and reading his words to them is the first time I thought, "Yeah, I'm going to need to marry this guy." So Ashleigh will always be a part of that memory, and so many other memories. She will always be a part of my life in so many ways.

This time, and losing Ashleigh so suddenly and unexpectedly, is obviously very hard, and I know we will continue to mourn her loss. However, I am also happy, excited, and relieved for her that she is free from all the burdens, limitations, sicknesses, and hardships placed on her in her time on earth. She was handed way more than any person should be asked to bear, and never complained. She was poked, prodded, pulled, lifted, carried, brought down, gradually lost skills and abilities, and the worst we ever got back from her was an occasional glare that said "can you please just leave me the heck alone". It is comforting to know that she is with Jesus, and she can walk again, can talk again, can see, run, play, sing, can understand, can comprehend, can communicate. She finally gets to be the person she was always meant to be, always should have been. I will miss her here with us, but I also very much look forward to the time when I get to see her again, and get to know the real Ashleigh as she was always meant to be."

Research Update

The Trials Continue

Jodie Gillon, Vice President, Patient Advocacy for **Abeona Therapeutics**, continues to update on the development of treatments, encouraging progress and enrollment opportunities in the trials for both Sanfilippo type A and B.

"We had the priviledge of hosting the **The McGee Family** in our Cleveland facility earlier this year. The opportunity to learn from families first-hand helps us design our clinical trials and education programs. Most of all, these interactions are a source for continued motivation as we get to see how every day counts. This was the first time we hosted grandparents and it was illuminating for us to hear the impact of not only witnessing your grandchild lose skills but the effect of watching your child suffer for years and the helplessness they feel."

Latest Data

On July 25, 2019, Abeona reported positive interim data from the Transpher A study. The data suggests that ABO-102 has a favorable safety profile and therapeutic potential for the treatment of MPS IIIA. Neurocognitive development was preserved in the three youngest patients enrolled in high-dose cohort 3 and they were tracking within normal age equivalent development 12-18 months after treatment. Robust and sustained biomarker improvements were seen across all dose cohorts, 8-24 months after treatment, and no product-related serious adverse events were reported in the study.

Abeona is currently enrolling eligible patients into Phase 1/2 clinical trials for MPS IIIA and IIIB

Abeona is conducting global clinical trials to assess the safety and efficacy of gene therapies for patients with MPS IIIA (Transpher A) and IIIB (Transpher B). To be considered for enrollment in the Transpher A or the Transpher B studies, potential subjects must be between 6 months to 2 years of age or ≥2 years with cognitive Developmental Quotient (DQ) of ≥60. A full list of inclusion/exclusion criteria for each study is available at ClinicalTrials.gov (MPS IIIA: NCT02716246; MPS IIIB: NCT03315182).

The Transpher A study is being conducted at clinical sites in the U.S., Spain, and Australia, while the Transpher B study has sites in the U.S, Spain, and France. Travel assistance is available for patients and their families enrolled in either study.

At Abeonatrials.com, you can find a six-question survey to see if your child may be eligible for the Transfer A study. If the survey results suggest that your child may be eligible for the study, you will have the option to send your information to the nearest clinical study site for evaluation by a study investigator. Completing the survey does not mean a child is eligible; the doctor overseeing the clinical study at each site (principal investigator) will still need to evaluate and decide if your child can undergo screening for enrollment in the study. Only after the full screening process is complete at the site will the investigator be able to determine if the child is eligible to participate in the study.

Study ABT-003 is enrolling patients with middle and advanced phases of MPS IIIA who may not be eligible for the Transpher A study. The clinical trial is evaluating a single-dose of ABO-102 that is delivered using an intravenously administered AAV9 vector. While there is no age requirement in this study, potential volunteers must have a cognitive DQ lower than 60 to be screened for enrollment. They must also be ambulatory, with or without assistance. A full list of inclusion/exclusion criteria for study ABT-003 is available at ClinicalTrials.gov by searching 'NCT04088734.' You may also consider providing your child's physician with this clinical trial identifier to help determine if they may be eligible for the study.

Reflections, Gratitude and a Life Filled with "Happy"

This KirbyGram is filled with the harsh reality for families such as ours. These children's lives and those who went before them are important to recognize and honor. It's part of what this Foundation has always done. Obviously, this year was personally devastating to our family. But, that being said, this Foundation's beginning and decades of success, were built on hope, hope that allowed our family to stand up from the devastation of Kirby's diagnosis and live our lives in the manner she so richly deserved - celebrating life and family and creating joy-filled memories. And now, we feel it is more important than ever to leave you this holiday season with that same joy-filled feeling, remembering our daughter in the light she so brightly showed for 28 years and ensuring we move forward in a manner that would bring that beautiful smile to her face.

Here are some of the happy feelings Kirby left with family and friends. We hope these words impact you, too, in some small way and most importantly, make you smile.

"She would look into your eyes, the eyes of her Mom, Dad and sister, her family, friends and visitors, and show them how much she loved them, without condition, without asking for anything in return, without saying a word. Just be, and be with me. Kirby gave us that gift of unconditional love. And isn't that the greatest triumph of any life? To love and be loved, without limit, without reason, without words."

"Kirby taught us the beauty of smiles, love and the strength of family."

"She was a force that changed all of our lives for the better. Even through the darkest of pain, she taught us how to love. She made us believe that we could find joy in absolutely every day."

"That's the thing about experiencing death - it allows you to experience the most intense forms of love…..And it's that love, cultivated by Kirby, that gives me the confidence that there will be lots of happy times ahead for all of us."

"She gave us strength and purpose. We do not know for certain how long any of us have here and we cannot foresee the trials or misfortunes that will test us along the way. But I will tell you what we can do now for Kirby's legacy is live our best lives with purpose and with love and most of all happiness. Let's use each day to show each other how much we care, and let's treat others with the kindness and respect that we wish for ourselves."

"She taught me so much about life and all the small wonderful things that should be celebrated each day. Thank you, Kirby, for helping teach my kids about acceptance, love and compassion for others."

"Kirby gave us the gift of perspective. Every day that Kirby was with us was a blessing. She reminded us that most of our problems are not really problems…be grateful for what we have…choose joy over gloom, because ultimately, life is so fragile."

"Kirby gave us the example of perseverance and inner strength. She confronted and fought battles that would have crushed many of us. She showed us what it is to be strong, because strength is not just found in being the biggest, the toughest, or the fastest. Strength is found in one who keeps going, keeps trying, day after day, and long after others give up."

"Simply put, Kirby refused to give up or succumb to changes she experienced by withstanding Sanfilippo and fought back with amazing fortitude. Strength is found in one who knows success isn't always big and flashy, but knows that sometimes the greatest success is found in the tiniest of victories."

"Hope is a tricky word. It is fluid. Enduring when you know to look for it. Sue, Brad, sister Maggie and nephew Anthony know hope. It is in their hearts, even today. Kirby put it there. The future, tomorrow, other families' hope is here because Kirby was."

"Because of Kirby: I am more appreciative and accepting of life's simple gifts; I've seen how big love can be; I've learned every child deserves every opportunity; I've learned to not worry about how to communicate, just do it and you'll find your connection to others no matter how different our lives are; I try to be ever present; I understand that it is possible to enjoy life, be happy and spread love despite your circumstances; and I know all the words to Barney's "I Love You.""

"My favorite day of those summers was always the first day back. Kirby Derbs would watch me for a few moments, and then I saw that moment. The moment when she saw me...The tilt of her head and that mischievous smile. I've lived lots of places, and I've met lots of people. People look, but not everyone really sees you. Kirby gifted that to me, and I am forever grateful. That tilt of the head, when she recognized whatever was inside me that was connected to her...I will cherish that forever. And I'm almost 40, but I still stomp through a puddle sometimes. Just because. And I always smile."

"Be in the moment, love those around you, hold each other close, and look for a greater cause. Choose to find joy in your life, and by all means, be happy. That is what Kirby stood for."

Kirby would ask, "Happy?" And she meant it. She wanted to know if we were just as happy as she was, because that was how she lived her life.

"Don't cry because it is over.
Smile because it happened."

— Dr. Seuss

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