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 2018

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 shared our thoughts on both the direction of the Foundation and our time with Kirby and how recent events had given us clarity. The priority given to our efforts and time spent has not changed. Our resolve for the Foundation and its mission remain. Our time with Kirby has never been more precious and treasured.

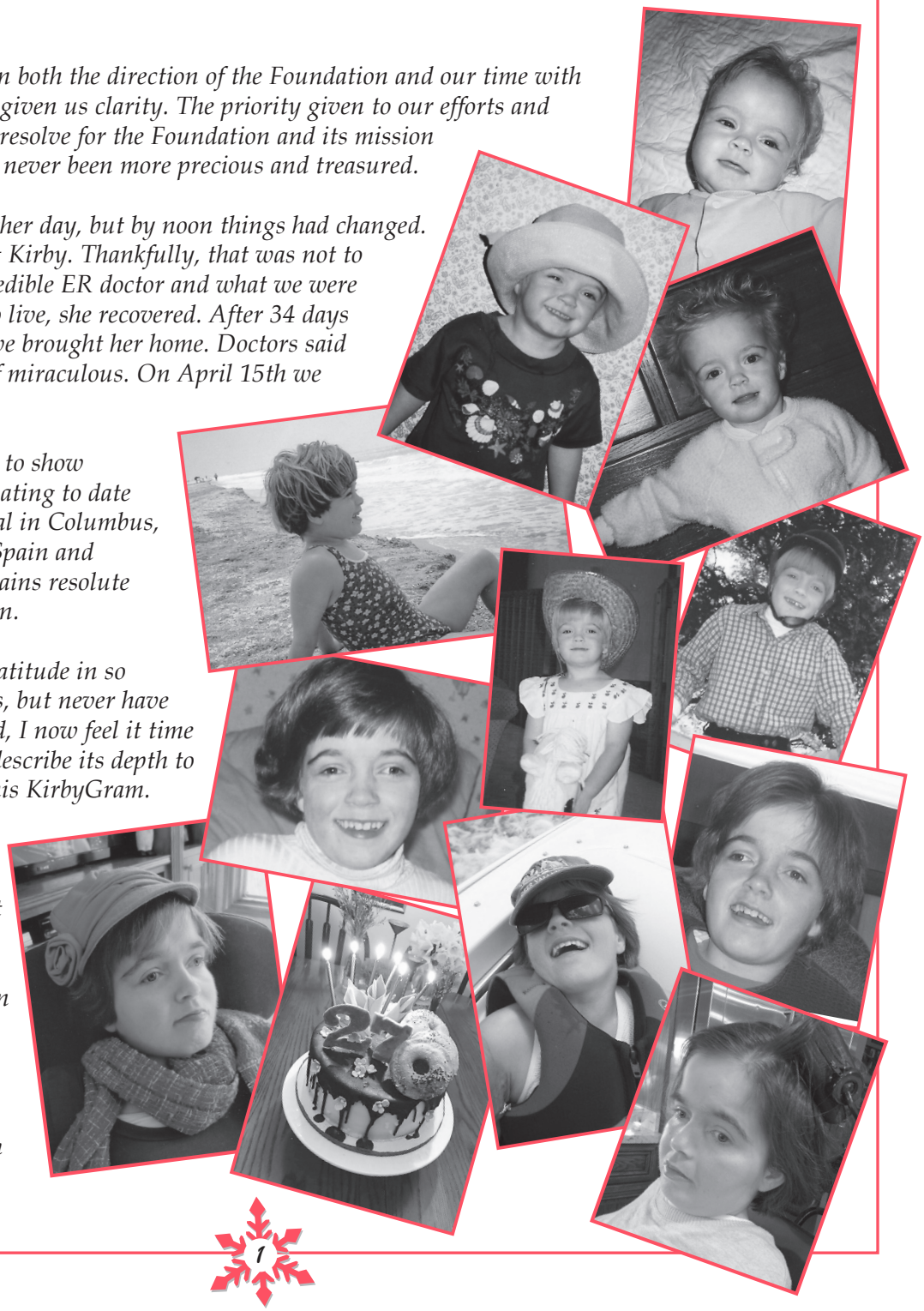
December 31st started like any other day, but by noon things had changed. Brad and I were faced with losing Kirby. Thankfully, that was not to be. Through the efforts of an incredible ER doctor and what we were told was Kirby's heart and will to live, she recovered. After 34 days in the ICU and step down unit, we brought her home. Doctors said her recovery was nothing short of miraculous. On April 15th we celebrated her 27th birthday.

The trials for Sanfilippo continue to show promise with 13 children participating to date at Nationwide Children's Hospital in Columbus, OH, and two additional sites in Spain and Australia, as the Foundation remains resolute in its efforts to include all children.

I know I have spoken to you of gratitude in so many ways over the past 24 years, but never have I felt it so wholly and deeply. And, I now feel it time to use this opportunity to try to describe its depth to you within the pages that close this KirbyGram.

We thank you for continuing to enable our dream of a cure for all children with your support of the Foundation as we spend our days with Kirby. The peace we feel within is a direct reflection of your support, commitment and compassion.

—Sue and Brad Wilson



Fundraising News

Facebook and #GivingTuesday

Molly and Kirby

For Kirby's cousin **Molly Krause**, there wasn't a second thought to using this opportunity to help. With her love for Kirby and her passion for Facebook, this fundraiser had success written all over it! Her many Facebook friends agreed and contributed $2,280 on last year's #GivingTuesday held November 28th.

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 your same account. The only difference is the smile on Kirby's face!

The Foundation Gives Thanks . . .

To Kirby's **Aunt Beth** and **Uncle John Wilson** for their contribution and to the **JPMorgan Chase Foundation** for its matching donation;

To Kirby's **Grandma Wilson** for her donation in fond memory of her longtime friend Veda English;

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 our secret **Facebook Friend** for donating to our mission;

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

To **Anthony Bernard** and **Americas' SAP Users' Group** for their donation to the Foundation;

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 $710 to date this year, your "hometown girl" thanks you from the bottom of her little heart;

To the many supporters who made a holiday contribution. Donations totaled more than $9,700;

To **Barbara Cummings** for her most generous contribution, and

To **Karen and Bill Rajki** for their donation in fond memory of our dear friend **Janice Collins**, who stood by Sue's side to do whatever necessary in assisting in the start-up of this foundation and beyond. She is missed by the many people whose lives she so graciously touched.

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

To "**Grandma and Grandpa Kidwell**" for their donation honoring **Brooke and Ashleigh Kidwell's** birthdays and to **Anna and Dave Kidwell** for their donation and years of support in helping with the Sweetheart Dinner Dance – all in honor of their girls;

Brooke

Ashleigh

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

To the **Manafort Family**, who honored 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 **Joann and Peter Smulski** for their donation in memory of Rhianna's grandfather, **James E. Couture**; and,

To the people who contribute to the **Artisan Shop's** cash jar, Rhianna's parents, **Cynthia and Gene Logan**, give their heartfelt thanks.

Fundraising News continued

A Note From Cynthia Logan

I recently saw a wonderful photo of a large group of MPS families. I shared it with my husband and said, "these are the NEW families." I could see his heartache as he looked briefly and in his own quiet place reflected on our journey with our beautiful daughter Rhianna. When we began our fundraising back in 1995, we created "Rhianna's Hope." Keyword was HOPE. Back then with only one researcher in the world working on Sanfilippo, we had to let hope be the driving force. So with lots of love and faith we embraced every fundraising opportunity brought to us by friends, family and even strangers. Our hope was blessed by the efforts of Brad and Sue Wilson and the strength of their daughter Kirby.

Today I follow the MPS community from a distance, as the heartache of losing Rhianna is still very powerful. I see the continued loss of these amazing children - each of them sharing a profound level of grace, strength and courage. When I saw the photo of the new families, I smiled because the hope for these children is very strong, treatments are being trialed and the reality of a cure is within reach. Our work is not done. Please pray and continue to support the fight for a cure. Share the stories and ask someone you know to donate. Gene and I thank those who continue to donate in memory of Rhianna. We send love and hugs to all the MPS families and the Wilsons.

Rhianna picking out a tree with her dad Gene and grandfather Poppi

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Lucas Montgomery
July 31, 1997 – July 22, 2018

Another beautiful soul lost. Our hearts are broken. But, our determination to cure must continue to be as boundless as our love for these inspirational children. They deserve no less.

The Montgomery Family

Lucas's family was a true powerhouse in advocacy and fundraising. It's energy inspired many, not only within the Sanfilippo community, but also beyond. Lucas's impact lives on.

In the words of his loving family —

Mom Stacey — "Your strength filled me with pride and gave me the belief that I was as strong as you in mind, body and soul. Faith always gets me through, knowing the day we meet again, my heart will be whole once again and my arms will no longer miss embracing you." …"You made me stronger, wiser and full of hope just with your smiles."

Dad Lew — "…As I looked to the sky and saw a shooting star, I realized it could have been him heading back to be with our God. So I smiled, as I often do when thinking of him, I gave a tear of happiness, and whispered like I did every night, closed my eyes and pointed to the sky and said, "I Love you Lucas." The stars seemed to shine brighter in that moment of the night sky and twinkle as his eyes did when we saw each other. Thanks li'l guy for your spirit and for making me so darn happy every single day. "

Sisters Chelsey, Hailee and Mariah — Let Lucas and his legacy be a lesson to us all. Always be kind to one another, whether you understand what a person is going through or not. Always help one another, because life is about bringing those around you up, not down. Smile often, even when the odds are against you. Be unselfish in your acts, and when you find yourself in pain, keep fighting and keep pushing forward, because it too shall pass.

Research Update

The Trials Continue

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

"ABO-102 (scAAV-SGSH), investigational gene therapy for individuals with MPS IIIA, continues in Phase 1/2 clinical trial and has enrolled a total of 13 children, of which 3 were in the lowest initial dose being studied, another 3 in the mid-range dose and 7 at the highest dose in the study. Enrollment continues across three sites that include Nationwide Children's Hospital in Columbus, OH, and Hospital Clinico Universitario of Santiago de Compostela, Spain, and Women's and Children's Hospital in Adelaide, Australia.

"Phase 1/2 clinical trial to investigate gene therapy product, ABO-101 (AAV-NAGLU) for patients with MPS IIIB is now enrolling in two locations, Nationwide Children's Hospital in Columbus, OH, and Hospital Clinico Universitario of Santiago de Compostela, Spain. Abeona plans to add clinical sites for the trial in three European countries, including France, Germany and the United Kingdom."

Most recently, Michelle shared news of Abeona's agreement with REGENXBIO.

"I'm pleased to share that we have entered into an agreement with REGENXBIO for world-wide exclusive license to NAV AAV9 for the development and commercialization of treatments for MPS IIIA, MPS IIIB, CLN1, and CLN3 diseases. NAV AAV9 is the specific viral vehicle used to deliver in our ongoing clinical programs for MPS IIIA and MPS IIIB and pre-clinical programs for CLN1 and CLN3. This is an important next step and additional demonstration of our commitment to the children and families of the communities that we are aiming to develop potential gene therapy approaches for.

"The achievement of these licenses is also a reflection on the early science that so many patient groups focused on Sanfilippo syndrome and Batten disease first supported."

Excerpts from Abeona's recent press releases on these developments follow:

November 5, 2018 — Abeona Therapeutics Inc. (Nasdaq: ABEO), a leading clinical-stage biopharmaceutical company focused on developing novel gene and cell therapies for life-threatening rare diseases, and REGENXBIO (Nasdaq: RGNX), a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy based on its proprietary NAV® Technology Platform, today announced a license agreement to REGENXBIO's NAV AAV9 vector for the treatment of four diseases: Sanfilippo syndrome type A (MPS IIIA), Sanfilippo syndrome type B (MPS IIIB), Infantile Batten Disease, also known as neuronal ceroid lipofuscinosis type 1 (CLN1 Disease), and Juvenile Batten Disease, also known as neuronal ceroid lipofuscinosis type 3 (CLN3 Disease).

"This agreement is an important milestone that underpins the therapeutic potential we see in our Sanfilippo syndrome and Batten disease programs featuring the NAV AAV9 vector, which have the potential to transform the lives of patients," said Carsten Thiel, Ph.D., Chief Executive Officer of Abeona. "Data from our clinical and preclinical programs and the success of the NAV AAV9 vector observed in other indications strongly position the platform as a leading technology for investigational gene therapies for the systemic and CNS manifestations of lysosomal storage diseases."

Under the terms of the agreement, REGENXBIO has granted Abeona an exclusive worldwide license (subject to certain non-exclusive rights previously granted for MPS IIIA), with rights to sublicense, to REGENXBIO's NAV AAV9 vector for the development and commercialization of gene therapies for the treatment of MPS IIIA, MPS IIIB, CLN1 Disease and CLN3 Disease. In return for these rights, REGENXBIO will receive a guaranteed $20 million upfront payment, $10 million of which will be paid upon signing and $10 million of which will be paid within 12 months of the effective date. In addition, REGENXBIO will receive a total of $100 million in annual fees, payable upon the secondthrough sixth anniversaries of the agreement, $20 million of which is guaranteed. REGENXBIO is also eligible to receive potential commercial milestone payments of up to $60 million. REGENXBIO will also receive low double-digit royalties on net sales of products incorporating the licensed intellectual property.

"This license agreement further validates the potential of NAV AAV9 for the treatment of systemic and CNS manifestations of lysosomal storage diseases, as well as the strength of our intellectual property portfolio," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "We are pleased to initiate our partnership with Abeona as they continue to advance multiple programs using NAV AAV9 through and toward clinical trials in indications with significant unmet medical need."

Sept. 12, 2018 — Abeona Therapeutics Inc. (Nasdaq: ABEO), a leading clinical-stage biopharmaceutical company focused on developing novel cell and gene therapies for life-threatening rare genetic diseases, today announced authorization to move forward with a Phase 1/2 clinical trial in Spain for the Company's gene therapy product ABO-101 (AAV-NAGLU) for patients with MPS IIIB (Sanfilippo syndrome type B). The clinical study was approved by the Agencia Espanola de Medicamentos y Productos Sanitarios and is being conducted at Hospital Clinico Universitario of Santiago de Compostela, Spain. This will be the Company's second clinical trial conducted in Europe, alongside the ongoing Phase 1/2 clinical trial for patients with MPS IIIA (Sanfilippo syndrome type A). Abeona first initiated this trial in the United States. Abeona plans to add clinical sites for the trial in three European countries, including France, Germany and the United Kingdom.

"The authorization of our ABO-101 trial in Spain is a significant milestone for children suffering from MPS IIIB, a devastating and deadly disease with no approved treatment options," stated Carsten Thiel, Ph.D., CEO. "We are encouraged by the preliminary results observed in our U.S. trial to date, both in clinically relevant biomarkers and in the ongoing safety profile, and are excited to bring this therapy to patients in Europe." Subjects in the Phase 1/2 trial receive a single, intravenous infusion of ABO-101, which uses an AAV vector to introduce

the functional NAGLU gene to treat patients with MPS IIIB disease. Subjects will be evaluated at multiple time points post-injection for safety assessments and efficacy parameters. The clinical program is supported by a Natural History Study which included potential efficacy assessments consisting of neurocognitive evaluations, biochemical assays and MRI data generated over one year of follow-up assessments.

ABO-101 has been granted Rare Pediatric Disease Designation in the U.S., and Orphan Product Designation in both the U.S. and the European Union.

About ABO-101 (AAV-NAGLU): ABO-101 is Abeona's first-in-human, adeno-associated viral (AAV)-based gene therapy for MPS III (Sanfilippo syndrome). Treatment involves a one-time intravenous delivery of a functioning copy of the N-acetyl-α-D-glucosaminidase (NAGLU) gene to cells of the central nervous system (CNS) and peripheral organs, with the aim of correcting the effects that result from the genetic aberrations that are the root cause of the disease. Following administration of a single dose in Sanfilippo preclinical animal models, ABO-101 induced cells in the central nervous system and peripheral organs to produce the missing NAGLU enzyme, which then restored underlying sugar (glycosaminoglycan or GAG) storage pathology to normal levels in cells. In preclinical in vivo efficacy studies in Sanfilippo syndrome animal model, ABO-101 demonstrated functional benefits that continue for months after treatment. A single dose of ABO-101 significantly restored normal cell and organ function, corrected cognitive defects, increased neuromuscular function and normalized the lifespan of animals with MPS IIIB after treatment compared to untreated control animals. These results are consistent with studies from several laboratories suggesting AAV treatment could potentially benefit patients with Sanfilippo syndrome. Safety and efficacy studies of AAV gene therapy treatments for Sanfilippo syndrome have recently been published in several peer-reviewed scientific journals.

May 31, 2018 — 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 opening of The Elisa Linton Center for Rare Disease Therapies, the commercial GMP manufacturing facility for gene and cell therapies in Cleveland, OH. The GMP facility will have the capability to manufacture clinical and commercial grade products over Abeona's multiple programs, including recessive dystrophic epidermolysis bullosa (RDEB) and Sanfilippo syndrome. The ribbon-cutting ceremony and first facility walk-through will be held today, May 31, 2018.

Elisabeth and Sue in Abeona's Founder's Room

"The opening of The Elisa Linton Center for Rare Disease Therapies is a momentous occasion and underscores Abeona's ongoing commitment to transforming patients' lives," said Carsten Thiel, Ph.D., Abeona's Chief Executive Officer. "Our development of internal manufacturing capabilities bolsters our position for commercial readiness as we continue to execute on our vision to bring these therapies to the patient communities that need them."

EB-101 is an autolgous, ex-vivo gene-corrected cell therapy where the COL7A1 gene is inserted into a patient's own skin cells (keratinocytes) for the treatment of RDEB, a rare and devastating skin disorder. ABO-102 is an adeno-associated virus (AAV)-based gene therapy in development at Abeona for the treatment of Sanfilippo syndrome type A (MPS IIIA). Both programs were recently granted the Regenerative Medicine Advanced Therapy (RMAT) designation by the U.S. Food and Drug Administration (FDA), emphasizing the unmet need for patients with RDEB and MPS IIIA.

In addition to the production of the EB-101 and ABO-102 therapies and the AIM™ AAV vector lab, the 6,000 square-foot facility will satisfy the necessary chemistry, manufacturing and controls (CMC) requirements for commercial development. The second stage of the Company's manufacturing strategy has been initiated with the construction of an additional 20,000 square-foot facility that will be used to further meet the anticipated commercial demand for development programs in the longer term.

This past May, the Wilsons attended the opening ceremony of Abeona's commercial GMP manufacturing facility, The Elisa Linton Center for Rare Disease Therapies in Cleveland, OH. Sue comments, "It was an honor for Brad and me to be included in such a momentous occasion representative of the strides being made toward a treatment. Seeing the space, meeting the people and learning of the processes was emotional, to say the least. We were grateful to be side by side with the Linton family for this bittersweet tribute to the memory of Elisabeth and Randall's dear daughter Elisa and to the many accomplishments made through their foundation, the Sanfilippo Children's Research Foundation."

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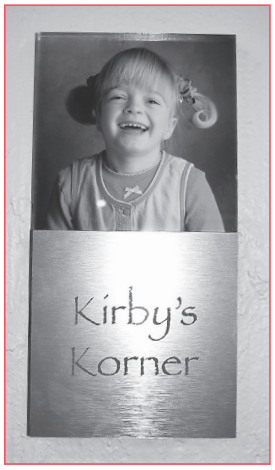
Wilsons, Lintons, and Abeona's Michelle Berg and Tim Miller



While there, the Wilsons also were able to see Abeona's meeting space named "Kirby's Corner." When first told of this, Sue was asked to think of something to say, keeping in mind what Kirby might think. Her response, "A place where happy and hope meet." They were also able to see the "Founder's Room," named after the company's 12 founding foundations, the Foundation being one. CMRF's statement reads, "These walls are filled with the vision of families worldwide who have joined together in hope for our children, determined to make a difference by telling our stories. Together we have created a formidable front against what we were all told was an incurable disease—all of us inspired by our children's unbounded spirit and tenacity."

Gratitude

When Brad and I think of this Foundation and its beginning, we are struck by the memory of our divergent emotions. As some of you may remember, the first words that were spoken to us after receiving the diagnosis of Sanfilippo on then four-year-old Kirby were: "There is nothing that can be done. Enjoy her while you have her. She is the same little girl she was yesterday." Maybe the doctor was right with that last statement, but that's where it ends. Yes, she was the same little girl that day that she was the previous, but there was so very much that could be done, thanks to Dr. Elizabeth Neufeld, our family, friends and the community of people that gathered and believed in the power of changing our devastation into our hope — a cure for Kirby.

It was Dr. Elizabeth Neufeld, Distinguished Professor Emerita, Biological Chemistry at UCLA, that explained to us the reason rare diseases did not get the attention of other, more prevalent diseases. Funding. But that did not stop her. This 1994 National Medal of Science honoree devoted the majority of her career to investigating Sanfilippo and potential treatments. Her research and mentorship in the area of lysosomal storage disorders, of which Sanfilippo is one, is the basis for work being done today. At 90 years old, although retired as Chair of the Dept. of Biological Chemistry, she still attends conferences, sharing her brilliance with others in the field. No matter where the cure and/or treatment comes from, we will always look at this woman as the person that enabled it all and gave us our direction.

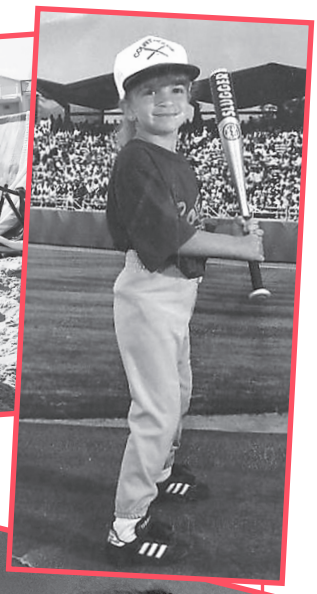
It was our family and a group of friends like family, that first wrapped their arms around us and to this day, stand steadfast by our sides. An incredible and unbreakable bond that has given us comfort in the midst of our fears or uncertainty and helped us to navigate our journey — always with unconditional love.

The list is seemingly endless when it comes to companies, organizations, merchants, churches, school groups and individuals that have rallied around Kirby, our family and this Foundation's mission of a cure. In 1995, news spread of what one reporter 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, to many, she was the girl next door. So you gathered and inspired our hope. A community creating a formidable front against what we were told was an incurable disease, proving what can be done, empowering brilliant research and giving hope to children afflicted worldwide.

When we think of the impact you all have enabled with the $4.25 million in grants that the Foundation has awarded, as well as your kindness throughout the years, we can't help but think back to that day in April, 1995 when we were told "There is nothing that can be done." In fact, there was so much that could be done, and together we did it and continue to, by the grace of God, with Kirby. The warmth we feel from your compassion and the effect your generosity has had and continues to have on the progress of this Foundation and its mission create a story we hope makes you happy – something most important to our dear daughter that we know would bring a smile to her face.

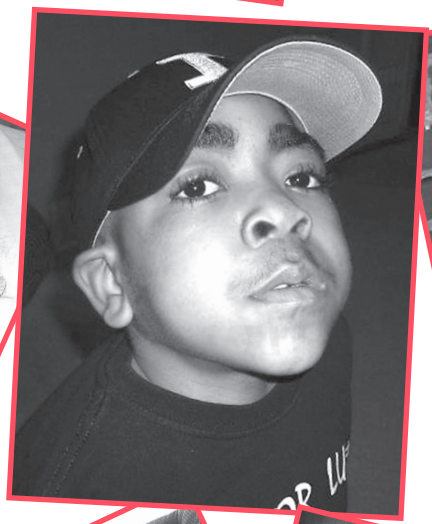
As we stand on the doorstep of what we hope is a treatment, we are honored to know you as our family, friends and a community of hope. Your bountiful blessings have eased the weight of our worry and continue to comfort us. Our profound gratitude runs deep and will never be forgotten.











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