

IMPACT REPORT

2022



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Dear Batten Community,

From its humble beginnings as a group of parents gathered around a kitchen table, to what is today a national organization advocating for treatments and cures, the BDSRA Foundation has navigated many twists, turns, peaks, and valleys. In 2022, BDSRA celebrated the 35th year of its journey, presenting the perfect opportunity to update the Foundation's identity to better reflect the organization's commitment to forward momentum.



Numerous people in the community were interviewed, focus groups were conducted, and the staff collaborated for more than eight months to capture the essence of BDSRA's brand in key messages. The new name for BDSRA – Batten Disease Support, Research, & Advocacy – Foundation better reflects the organization's pillars, demonstrating it is no longer an association. It defines where the organization should go. Leadership and the community agreed that no matter how support or research is provided, it is always an act of advocacy for Batten disease, so we are all Batten Advocates for a Cure.

Over the years, BDSRA conferences brought families from far and wide together to share their experiences. This tradition was stunted by COVID-19, but in 2022 our families came together again, in person and online. The first-ever hybrid conference hosted 440 people from around the world to envision A World Without Batten. Batten families, researchers, clinicians, and industry partners journeyed to Cleveland, Ohio, in July or joined electronically to celebrate, learn, and meet new friends. It was good to be together again!

The pursuit of research has always fueled BDSRA's journey. This quest has led us to attend numerous research-focused conferences and to fundraise to award research grants. Over the last several years, however, the medical research industry struggled as COVID consumed the majority of the world's Personal Protective Equipment. Supply chain shortages and new cleaning protocols proved both costly and time-consuming for the industry, halting most research and experiments. As a result, BDSRA also paused on awarding new research grants. We did award extensions to grants issued in 2019 and that research will soon resume.

Research is essential to creating A World Without Batten. Though experiments were paused due to COVID-19, the Foundation continued to focus on ongoing research and made that information more accessible, amplifying Batten voices. In 2022, Foundation leadership met with industry partners regularly to learn about their plans for and progress toward treatments and cures. We shared that information with our community and gave our partners dedicated space to publish updates every month in the Illuminator.

When the market proved hostile for some, we initiated a global response with our international partners and experts to help us understand what it all meant and could mean moving forward. I invite you to visit our YouTube Channel to watch it at your convenience. We attended countless rare disease conferences to learn and share what was presented there.

Advocacy has always been part of the BDSRA journey, but through the Foundation's strategic plan, it was elevated and given goals. The Batten Family Needs Assessment presented in 2022 helped us better understand our community and how we should advocate for your needs. Thank you for participating!

The board created an Advocacy Committee and, while it was virtual, we participated in Rare Disease Week and met with legislators throughout the year on three occasions to advocate for important legislation – including newborn screening and the STAT Act. Foundation members also attended conferences to advocate to researchers and clinicians who are unfamiliar with Batten disease.

In 2022, the Batten story was told with videos, podcasts, and panel discussions. Resources were developed to help our community share with educators and clinicians, including Toolbox Tuesday, a weekly social media post that we encourage our community to share. Patient advocacy will continue to be at the center of all we do at the Foundation to serve you, our community.

For everywhere that 2022 took me, my word is *Gratitude*. Thank you for allowing me to walk with you on this journey. Every opportunity I've had to meet you and your children and hear your stories has been a gift. The Foundation team and I will continue to move forward to advocate for the treatments and cures so desperately needed and to know and respect your word for your Batten journey.

With Hope,



Amy Fenton Parker
President & CEO



BATTEN COMMUNITY REUNITES AT 2022 ANNUAL FAMILY CONFERENCE

BDSRA Foundation's Annual Family Conference brings the Batten community together for a weekend of reunions, introductions to first-time attendees, education, and the opportunity to create memories.

The 2022 Annual Family Conference held at the Hilton Cleveland Downtown in Cleveland, Ohio was the first-ever BDSRA hybrid conference and the first to accommodate in-person interaction since 2019.

The time gap between the pre-COVID conference and the 2022 conference led to surreal interactions within the Batten community over the weekend. New families were introduced to the community, while multiple-time conference goers finally got to have long-awaited catch-up conversations, and other families were attending the conference without their loved ones with Batten by their side for the very first time.

Gina Hubsch, a first-time conference attendee in 2022 and Batten mother to Scarlett, admitted she pictured a dreary atmosphere prior to attending due to the severity and reality of a rare, fatal disease like Batten disease.

What she experienced was quite the opposite! She found herself instead taking comfort in the fact that she was surrounded by fellow parents who were on the same journey, learning and laughing with her new peers in the process. At the same time, Scarlett stole the hearts of attendees whether she was in childcare or taking walks with fellow Batten parents during mealtimes.

"It was nothing but love from the second we walked in the door," Hubsch said. "My biggest takeaway is you're not alone. People are just like you...I went in there seeing a bunch of beautiful, amazing, funny, active kids that were living the best lives that they possibly could at that moment, and truly, it was nothing but joy."

That joy was shared, thanks to the connection between Gina, Scarlett, and their newest friend, bereaved Batten father, Brock Benroth. Gina was overwhelmed upon arriving at the conference with Scarlett and was introduced to Benroth by BDSRA Foundation President & CEO Amy Fenton Parker.

"I just absolutely fell in love with Scarlett," Benroth said. "(Scarlett) reminded me of our first conference and the state that (my daughter) Kate was in where she could still walk, but she was super herky-jerky, and you had to kind of hold her hands and walk with her."

Kate passed away in 2017, but Benroth still attends the conference to lend a helping hand to first-time attendees who find themselves overwhelmed, just like he and his wife, Ellie, once were.

Benroth never forgets the courage of the bereaved parents who assisted them. “To me, it was awesome to see (bereaved families) there and to see that there’s kind of a light at the end of the tunnel,” Benroth said. “It was just really nice to see them and see that it’s going to be OK. It’s a rough journey, but everything will work out, and it’ll be as OK as it’s going to be anyhow.”

“I hope that I can convey that to other fairly newly diagnosed parents,” he added.

Attendees spent their time participating in research, advocacy, and/or care sessions, catching up, taking care of their loved ones, or spending time at the Cleveland Botanical Gardens through the Life Goes On program for bereaved parents and caregivers.

The BDSRA SIBs program also paved the way for more memorable times for siblings of those affected by Batten disease. The 2022 program included swimming, roller skating, and a trip to the zoo.

“The younger SIBs – to see their reactions every year and to see their excitement to want to come back...just makes me feel so good to know that I’m able to help these younger kids, the same way that the older SIBs, when I was a kid, planned and hosted and did everything needed for the conference,” SIBs leader Jeni Montavon said. “To come to a conference and to meet people that are going through the same thing you’re going through is something you can’t get anywhere else.”



Restricted Gifts

\$415,311

Batten Family
Help Grants

6

Equipment
Exchange Grants

3

Fore the Journey
Grants

11

Virtual
Program
Registrants

296

Annual Family
Conference
Registrants

440

Total
Family
Grants

\$27,608

RALLYING FOR RESEARCH: DONATIONS PAVE WAY FOR BDSRA RESEARCH POSITION

What started with a generous \$5,000 donation soon grew into a spontaneous give-a-thon during the 2022 BDSRA Annual Family Conference's banquet celebration in Cleveland. There was a singular goal: to secure funds to hire the Head of Research & Medical Affairs position at the BDSRA Foundation.

Dr. David Pearce, President of Innovation Research & World Clinic at Sanford Health and a BDSRA Board Member, provided the lead gift of \$5,000. Not long after, Wayne Kiefer, a bereaved Batten father, (and now a BDSRA Board Member) and Larry Killen asked to lead the Text2Give® campaign to help fund the research position.

Kiefer's daughter, Ashley, was diagnosed with CLN2 in 1996. He then attended his first conference three weeks after the diagnosis. Larry and his wife, Jean, bereaved parents of Bret, were among the first people they met.

"I was welcomed with open arms by the Batten community," Kiefer said. "Even though Ashley passed away in 2002, the people I met were like family, and we always reunited at the conferences. It is a strong bond we share."

Soon after Kiefer and Killen took the lead of the campaign, a barrage of incoming donations resulted in frequent interruptions of the music and dancing to update everyone in the ballroom on the amount of money being raised. This moment cemented the exhilaration of the organization's first in-person family conference since 2019.

"I have always known our community to be generous," Kiefer said. "While I wasn't surprised by others wanting to help, I was humbled by the amount of dollars raised by so many different people and organizations at different points in their life's journey against Batten in a short period of time."

"I am so incredibly grateful to be associated with these types of people. Many who have lost their children, still show up, and fight and donate to honor the memory of our beautiful angels."

Donations were given in cash, checks, credit cards (through the website), and Text2Give.® Donors also secured company matches and funding for their volunteer hours. Drew's Hope, a non-profit foundation established in 2007 by Board Member Tony Ferrandino and his wife, Katie, in honor of their late son, Drew, committed to giving \$10,000 per year for three years.

“I was overwhelmed by the spontaneous support. To me, it was an affirmation that our community has complete buy-in for the vision set forth by the 2018 strategic plan,” BDSRA Foundation President & CEO Amy Fenton Parker said. “I am so grateful to everyone at the conference who contributed and the initiative of Wayne and Larry to make it happen.”

Overall, it was estimated that more than \$42,000 was raised as a result, including pledges.

“It was an electric night with a lot of positive energy that I hope we can repeat again,” Kiefer said. “I want to thank everyone who gave something that night, it shows that while one person may not be able to make a difference, many people can make a difference.”



RESEARCH

Restricted Gifts

\$297,251.38

Science Officer Initiative **\$44,043**

Ask-An-Expert Research Edition **4**

Research Conferences **4**



STRONGER TOGETHER: THE STORY OF BATTEN MOTHERS WHO TEAMED UP TO SPEAK TO THE FDA



In the early months of 2022, a Batten storm was brewing. Cristina Rosa-Vargas, mom to Juju, had sent an email directly to the FDA. Amanda Beedle, mom to Annabelle and Abigail, later requested a meeting via the Patient Listening Session (PLS) form through the FDA. Many other CLN2 parents followed suit, sending the PLS requests. The communications caught the attention of the FDA and led them to grant one meeting through the consolidation of the initial two requests.

With the tagline “Pipeline for a Lifeline,” a wide range of parents spent hours preparing to tell their collective stories. They wanted to express their absolute gratitude for the current treatment available to their children, but they also wanted the FDA to know they needed more. BDSRA Foundation worked behind the scenes helping the families refine their story and develop their ask of the FDA.

On March 17, the group assembled included Cristina Rosa-Vargas, Brian and Estela Rosko, Bekah Bowman, Michelle Breen O’Leary, Corrin Jackson, Amanda Beedle, and Suzette James. Other support came from BDSRA, Dr. Wang (CHOC), Rare Advocacy Movement, and Stephen and Sandra Lehrman, CLN2 grandparents of Chase and Courtney. They discussed barriers to additional treatments, the need for faster treatments, and better technology regarding animal models. They asked that BDSRA be granted the PFDD (Patient-Focused Drug Development) requested by a collective group in December 2021. They spoke from their hearts and gained feedback from the FDA in the following weeks.

A collective effort ensued between Jackson, Beedle, James, and Fennell that stretched across four states and three different time zones to create a presentation – with each CLN2 Batten mother presenting in each of the four sessions of the FDA CBER OTAT Patient-Focused Drug Development Listening Meeting – Patient Perspectives on Gene Therapy Products on November 15, 2022.

“I felt it was a really seamless collaboration between all of us, and it feels really good to be in that mix of people where you all know where each other is coming from,” James said. “And these women are incredibly smart and amazing and have (huge hearts).”

Battling through technical difficulties, James presented during the first session, “Patients and caregivers understanding of gene therapy risks and benefits.” She emphasized that the risk of not doing anything far greatly exceeds the risk of doing something.

Jackson followed James and presented during the second session, “Patients and caregiver involvement in clinical study design and execution.” “I basically wanted to inform the FDA that the patients and the families’ voices are not heard when designing a clinical trial,” Jackson said.



“Current tools and methods to capture patient-reported data and any existing challenges or gaps to capturing patient experience data” was the title of the third session, through which Fennell highlighted ways that CLN2 families experience data sharing – such as surveys, clinical testing, and panels.

“It was a little tricky because our community does not have a lot of sophisticated or comprehensive tools to do this,” Fennell said. “I think part of our learning experience listening to the other presenters was that the CLN2 community and maybe even the Batten community, in general, is really way under-resourced in doing this particular thing.”

Beedle capped things off for the group in the fourth session, “Approaches to leverage existing tools or opportunities for unique tools to capture patient experience data in gene therapy studies.”

She voiced that placebo studies and sham ports are unethical in a vulnerable population like those affected by Batten disease and that, as parents and a community, they would welcome the careful collection of data.

“As a community, we don’t want to invest all that time in collecting data only to later have it just be discarded,” Beedle said. “Time is of the essence and in many cases, our child really is his or her best control in looking at the setup of these studies.”

The entire session was the culmination of many Saturdays the four spent collaborating with each other and the hard work it took each of them to capitalize on the opportunity to make not only their own voices heard, but the voices of the entire CLN2 community.

“Advocacy means everything to me,” James said. “Whether it is making certain that I pack a type of lunch that my daughter can now access because she can no longer feed herself by mouth, whether it means making certain that she still gets reading instruction, whether it means speaking to the FDA on a Wednesday afternoon, or working with the BDSRA – it means everything.”

Time is of the essence, and stopping isn’t an option as the realities of this fatal disease are apparent.

“Batten disease takes everything from our children,” James said. “Advocacy touches every part of your life, and every piece of that advocacy is as critical as the next.”

ADVOCACY

Restricted Gifts

\$22,354.32

Illuminator
Donations

90

New
Illuminator
Subscriptions

120

Advocacy
Meetings

17

States Added
RUSP Alignment

4

International
Batten
Awareness Day
Participants

416



LEGACY GIVING MAKES A LASTING IMPACT

Betty (Cornwell) Wetherbee grew up in a large family in Illinois. She and her sister, Myra Lou, were the younger ones. As children they were very close. They eventually grew up and got married, and their journeys through life were quite different.

Vernon H. Wetherbee was adopted and later assumed his given name, Joe. He was a gunman in World War II and then went to the School of the Art Institute of Chicago, graduating with a Bachelor of Art Education Degree. He met Betty, the love of his life, and together they moved to Colorado.

Joe continued his studies at the University of Denver, obtaining a Master of Arts Degree. He established the first Art Department at Wheat Ridge High School in Jefferson County, Colorado in 1950. Joe taught there for 33 years, retiring in 1983. Betty worked for the telephone company, and they continued to support the arts program in the county's schools.

Meanwhile, Betty's sister Myra Lou started her family back home in Illinois. Although they were heavily involved with children through the school system, Joe and Betty were never able to have children of their own. But Betty and Joe were close with Myra Lou's family, celebrating college graduations, weddings, and births.

Betty grew especially close to her niece, Myra's daughter Judy, and was always available for the major events in her life's journey. When Judy gave birth to her first child, David, Myra Lou became a grandmother – and Betty enjoyed the benefits of having a great-nephew. If the parents had an engagement and the grandparents were unavailable, Betty was all too happy to take over and help with the new grandson.

Betty treated Judy like her own daughter and David like her own grandson. On one visit in 1977, Dave remembers fondly that she took him to the Field Museum in Chicago for “The Treasures of King Tutankhamun” exhibit.

Betty also celebrated the birth of Judy's first grandchild, Clifford, her great, great nephew, son to Dave and his wife Corrina. Betty stayed connected to Dave and his family long after his mother, her beloved niece, passed away.

In 2003 shortly after Judy's funeral, Clifford's vision became a severe problem, and David and his wife were sent to an ophthalmologist in Chicago. Fortunately, their diagnosis journey was swift: Clifford had Batten disease (CLN3). The family attended their first family conference in 2004.

In 2007, Betty and Joe moved to Joe's hometown, Waupaca, Wisconsin to be closer to family. Dave and Corrina had recently moved to Springfield, Illinois, so they loaded up the family and went to visit Dave's great aunt and uncle. Sadly, it would be the last time they would get to be together, but they kept in touch. Joe passed in 2009, leaving his bride of 65 years.

In the world of philanthropy, Betty was not well-known to BDSRA, but she was clearly made aware of us through her great, great nephew's battle with Batten disease. She donated to BDSRA during her lifetime, but her biggest gift was yet to come.

Just after Christmas 2022, BDSRA received a six-figure gift from Betty for support and research of Batten disease. Betty had passed on February 20, 2020 and had left this bequest to the Foundation.

We regret that we weren't able to thank Betty ourselves, so we want to express an abundance of appreciation for the impact this gift will have on our Batten community.

Bequests of every value are meaningful to BDSRA, but we would like to be able to express our appreciation while you are living. If you or someone you know is making a will or estate plans and BDSRA is included in any way, please alert us so we can express our gratitude. If we can help you with the planning, let us know.



SUPPORT OUR MISSION

Greatest Need

\$228,817.75

5 fast things you can do:

DONATE

\$24,487

IRA Rollovers, Donor Advised Funds, & Trusts

ADVOCATE

\$49,136

Bike Events, Bowl-A-thons, & Batten Awareness Day

GIFT

\$33,467

Anniversaries, Weddings, Birthdays, & Tributes

EMPLOYER MATCH

\$12,015

Ask your employer about matching your charitable donation.

SHARE

Share your story to raise funds and awareness for Batten Disease.

Follow us, like and share our posts on Instagram, Facebook, X, and LinkedIn.



BOARD CHAIR ADDRESS TO COMMUNITY

Dear Batten Community,

This year we are asking our members how they would describe their Batten journey with one word. What a challenge. I think it depends on where you are on your journey.

When we learned our girls were diagnosed with CLN3 Batten Disease, my one word would have been “unbelievable.” As the years passed, I could believe that Batten didn’t exist, and my one word would have been “hopeful.” Fourteen years ago, when we lost our girls, my one-word journey turned to “loss.”

Like most of you, my journey is not over. It has been full of ups and downs, and through it all, the one bright place has been with the people I have met in the Batten community. Now my one word is said with pride, “family.”

There are so many others who have shared our experiences, and I am sure their one-word journey would sound like ours. My hope is that you find comfort, hope, and peace with someone inside our Batten family.

May God bless your family and grant you peace and strength.



Darlene Royalty,
Chair of the BDSRA Board of Directors





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