



Impact Report

2023

Batten Disease
Support + Research + Advocacy

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Dear Batten Community,

The year 2023 was a blur, but your BDSRA Foundation team speed-walked through the year with agility! We pivoted in the first quarter to share the responsibilities of the vacant support and advocacy position. That position remained open, and we are grateful for board members and volunteers from our community who continued to help us fill the gap.

In the first quarter of 2023 we welcomed four new board members who brought unique talents the current members sought. We also empowered the board's Support Committee, comprised of many community members, to drive the Annual Family Conference. They launched the first community contest to create the event theme, and in July convened in Denton, Texas, to "Give Batten the Boot."

In February, Patrick stepped in to represent the community during Rare Disease Week on Capitol Hill. He shared information and valuable details daily on social media.

One of the year's biggest wins came in the second quarter of 2023 when we added Ineka Whiteman, Ph.D., to our team as the long-awaited Head of Research & Medical Affairs. Ineka hit the ground running by chairing the Science and Research committee for the conference. It was there that we launched the BDSRA Family Register and announced the re-invigoration of the Batten Disease Clinical Center of Excellence program.

By the end of the second quarter, applications from around the country totaled 11, and BDSRA was overwhelmed by the response. We took it in stride, hosting the first meeting of the group during which the collective committed to developing standards of care and working together to reimagine the program.

Also in the second quarter, International Batten Disease Awareness Day trampled our expectations! We created the first-ever T-shirt design contest, and our community responded with nearly 20 designs and 100 votes. Board members secured a state proclamation and federal resolution to declare June 9 International Batten Disease Awareness Day. We raised the goal several times, and our community walked all over it with 35 teams, 365 individual participants, and 817 donations totaling more than \$52,000.

The pandemic was not kind to the biotech industry. The first announcement of a departure from Batten disease came in January of 2023, and many more followed. The BDSRA Foundation marched on, however, joining our global partners to continue advocating for treatments and cures. Our 2019 research grants came out of hiatus and recruitment for many other studies followed.

After securing grant funding, Ineka and I were able to join the International NCL 2023 conference in Hamburg, Germany. We attended a Patient Advocacy Organization meeting to present our organizations, share best practices and ideas. We had several meetings with researchers, clinicians, and families alike, from around the globe. We spent hours watching the presentation of many promising research findings, and we reported back what we could.



Throughout the entire year Linda managed more than 40 family grants, and Noah automated most of our processes to increase efficiencies, accuracy, and speed. Expanding our services meant we needed to create a steady operations income for BDSRA, so we launched Fam Funds and a crowd-sourced funding effort: \$5 Fridays.

Fam Funds were created as a way for families to share their stories, advocate for Batten disease, and invite others to donate in support of each family’s favorite pillar – Support, Research, Advocacy, or Greatest Need. Providing multiple ways to support BDSRA helps us to create the impact we desire for the community.

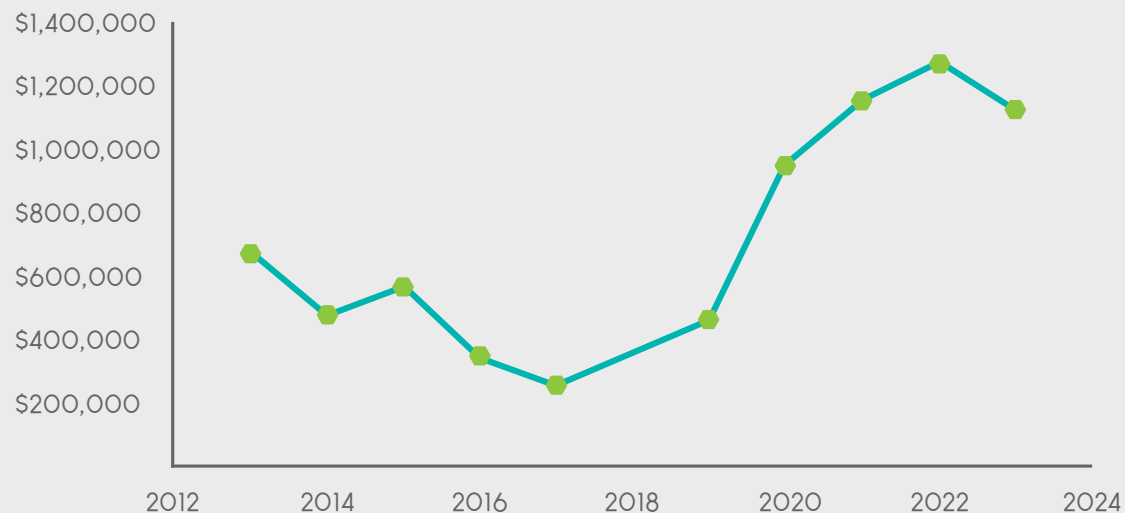
From Rare Disease Week and International Batten Disease Awareness Day to an energized Annual Family Conference in Denton, Texas, the BDSRA community and Foundation took big steps forward together. We made an impact, both nationally and globally. But we must continue walking forward to encourage and support all Batten families, fund and facilitate research, and advocate for treatments and cures.

Your Batten Advocate,



Amy Fenton Parker
President & CEO

HISTORY OF SUPPORT



BDSRA CENTER OF EXCELLENCE PROGRAM UNDERGOES REVITALIZATION



A BDSRA Foundation Designation
Center of Excellence

If you build it, they will come.

The famous line (“If you build it, he will come” in the film) from the 1989 film *Field of Dreams*, starring Kevin Costner and the late James Earl Jones, was once said during a BDSRA Centers of Excellence meeting.

Rather than building a baseball field in Iowa, though, Batten Disease Clinical Centers of Excellence were being revitalized, as announced at the 2023 BDSRA Annual Family Conference in Denton, Texas. The Dream was to establish regional centers where Batten families could receive full care and see multiple specialists at the same place.

“Over the many years, BDSRA has recommended Centers of Excellence, and we kind of lost our way with that for a number of reasons,” Dr. David Pearce, a BDSRA Board Member and Chair of the Science & Research Committee said. “And it became increasingly obvious that with our children, we need to have specific hubs where we can recommend to families.”

“This is a place where all the different sub-specialties, all the different types of physicians and doctors that your child may need to see are in one place. That’s so important,” he added.

The roadmap to revitalization began in late 2022 when BDSRA President & CEO Amy Fenton Parker met with Pearce, Dr. Jon Mink, BDSRA Board Member Barbara Wuebbels, Dr. Kourtney Santucci (Children’s Hospital Colorado), Dr. Scott Demarest (Children’s Hospital Colorado), and virtually, Dr. Raymond Wang (Children’s Hospital of Orange County, California) at the NCL Translational Conference in Lombard, Illinois, just outside of Chicago.

The pieces fell into place in the following months, including a six-figure gift from the late Betty Wetherbee a month later, and a concentrated effort was put into place for 2023. Applications were made available shortly after Pearce’s announcement in Texas.

At first, Parker, BDSRA Head of Research & Medical Affairs Dr. Ineka Whiteman, and others in ensuing meetings initially thought they would get four to five applications from academic centers and hospitals.

They were wrong.



Scan to learn more about
our Centers of Excellence.



SUPPORT

Restricted Gifts

\$349,565

Batten Family Help Grants

9

Equipment Exchange Grants

6

Fore the Journey Grants

14

Annual Family
Conference Grants

14

Family Party Registrants

175

Grief Chat Registrants

29

Annual Family
Conference Registrants

334

Total Family Grants

\$71,806

There were 11 applications submitted, which solidified that the Batten community has far more clinical support than anyone realized.

“We asked for families to encourage their own physicians, and it was clear that their input was working,” Parker said.

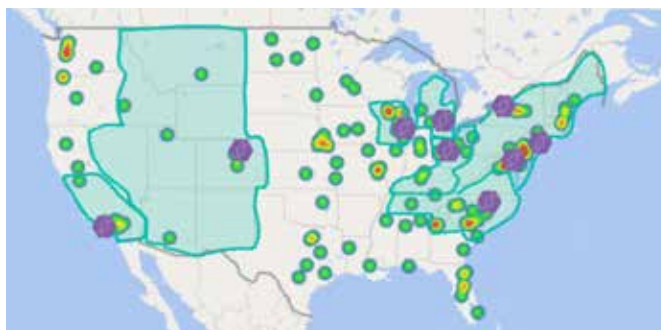
The application pool prompted a Centers of Excellence meeting on November 10, 2023. Whiteman quickly rolled up her sleeves to prepare the framework from which BDSRA could tab clinicians to seek their input on building the program, including the designation criteria, the four pillars, and what they believed is best for families and the standards of care.

“We pivoted in our approach and invited those Centers to help us co-design a national, stratified, and inclusive model,” Whiteman said. “It was clear from the outset that we have a genuinely collaborative and dedicated group of clinicians who share our vision – to ensure all our Batten families, regardless of where they live, have access to comprehensive, best-in-class care for their affected loved ones.”

This meeting would propel BDSRA into 2024 with a clear vision for the program. These regional centers would be established across the country so they can be more accessible for Batten families no matter where they live in the United States whether it’s for appointments, clinical trials, or consultations, ensuring Batten families receive access to the care they need.

“(The Centers of Excellence) will give us the hubs and places where we can have these clinical trials, and our children won’t have to travel long distances to participate in those trials,” Pearce said. “We want to put this in place because there will be a change in the environment in biotech technology. These therapies – that large number that we were talking about a couple of years ago – it will bounce back.”

CENTERS OF EXCELLENCE, U.S. FAMILIES, & REPORTED CATCHMENT AREAS



Key

 - CoE Location

Individuals Affected



DR. INEKA WHITEMAN MAKES IMMEDIATE IMPACT FOR BATTEN COMMUNITY & BDSRA

What began as a spontaneous give-a-thon during the 2022 BDSRA Annual Family Conference's banquet celebration in Cleveland, blossomed into a new reality long-desired by the BDSRA Foundation, BDSRA's Board of Directors, and the Batten community in 2023.

In June 2023, BDSRA officially announced Dr. Ineka Whiteman as the organization's Head of Research & Medical Affairs, overseeing the Foundation's research investment and grant process for all Batten disease CLN types to ensure the organization fosters and supports the best possible research into Batten disease in the United States and around the globe.

Dr. Whiteman's role also includes exploring and identifying opportunities for new research avenues and partnerships and working with industry partners on research pipelines and clinical program opportunities. Batten families could receive full care and see multiple specialists at the same place.



"Since the strategic plan was created in 2018, it was clear the BDSRA Foundation community wanted a science officer," President & CEO Amy Fenton Parker said. "We were very happy to quickly secure the rest of the funding needed to keep Ineka on with us for three years."

The year proved monumental for BDSRA as Dr. Whiteman helped rebuild and reshape the organization's science and research programming and communication.

One of Dr. Whiteman's first tasks was to plan the research session at the 2023 BDSRA Annual Family Conference in Denton, Texas, alongside Dr. Jonathan Cooper and Dr. David Pearce.

What followed was a cohesive series of presentations on how Batten disease research journeys from lab bench to bedside, from the Basics & History of Batten Disease (Dr. Keigo Takahashi), Models of Disease (Dr. David Pearce), Learnings from NCL Models (Dr. Jonathan Cooper) to application of Experimental Therapy Approaches (Dr. Michelle Hastings). This "dream team" laid a perfect foundation for the Clinical Trial Overview and Update, where Dr. Whiteman dove right in with the latest developments in more than 10 clinical programs.

The session was amongst the highest-rated at the conference, according to the post-conference survey.

"I enjoyed the lab-to-trial session because it was interesting to see the hurdles involved with getting to trial and the importance of natural history trials," an anonymous conference attendee wrote in the post-conference survey.

RESEARCH

Restricted Gifts

\$108,950

Total Research Webinar Registrants

201

Research Webinars Hosted

2

Family Register Enrollees

134

Research Conferences Attended

8

Next, Dr. Whiteman aided the launch of the BDSRA Family Register and Center of Excellence program and began writing a monthly column for BDSRA's newsletter, The Illuminator, detailing the latest research and clinical updates as well as research opportunities and publication summaries.

Whiteman served as a leading and reliable voice for the Batten community, offering valuable insight and breakdowns following clinical trial news and hosting BDSRA Ask-An-Expert webinars with researchers and clinicians.

Dr. Whiteman hosted two of these webinars in 2023, which garnered 201 total registrants. The first webinar featured news on REGENXBIO's Batten disease programs, and the second was a recap of Batten disease research from the year alongside a panel of clinicians and researchers.

"Partnerships and authentic collaborations and relationships are really central to this role, too," Dr. Whiteman said.

A crucial, longtime-coming call has finally come to fruition at BDSRA with Dr. Whiteman's hire, thanks to the help of donors. Dr. Whiteman's impact has already been felt throughout the organization and community and symbolizes yet another step toward achieving BDSRA's long-term vision of a world without Batten.

"I do feel genuinely blessed to be working alongside this incredible community every day," Dr. Whiteman said.



RARE DISEASE WEEK 2023 OFFERS A ROAD FOR OPTIMISM



Patrick Kotnik, BDSRA Marketing & PR Coordinator, attended Rare Disease Week on Capitol Hill in 2023. The following is a summary of his experience written by Patrick himself.

I didn't know what to expect when attending my first Rare Disease Week in 2023.

Although I was new to Batten disease advocacy, I knew how important it was for the Batten community to be represented and have an additional voice within other rare disease communities and on Capitol Hill.

I wasn't alone, thankfully, as I was joined by Donna Fogle and Sharon King, two bereaved Batten mothers, and advocates who each have lost a daughter to CLN1 disease. But when I arrived in D.C. and participated in the week's activities, I quickly realized the Batten disease community isn't alone in the fight for treatments and cures. We're in the same position amongst fellow rare disease communities.

Through all the sessions and networking, such as Rare Disease Day at NIH and the Legislative Conference the next day, I saw and met others advocating on behalf of rare disease communities I had never heard of – which is something Batten community members know all too well.

Yes, explaining Batten disease to countless people who have never heard of it and lack understanding of its severity and the desperate need for treatments and cures is tiresome but necessary. Capitol Hill is where change happens, and persistence is key to changing how rare diseases and clinical trials are viewed and handled in the U.S.

If you ever attend Rare Disease Week, you will be as overwhelmed the first time as I was. There's a lot of information from numerous presentations. But fear not, the EveryLife Foundation for Rare Diseases does a phenomenal job of breaking it all down (through small groups by state and lots of one-pagers) and preparing you for your meetings with legislators on Capitol Hill.

I had two meetings – both with Ohio representatives in the House. My first was an in-person meeting alongside fellow rare disease advocates with a staffer from Rep. Troy Balderson's office and a virtual one-on-one meeting with a staffer from Rep. Joyce Beatty's office.

The EveryLife Foundation provided four legislative asks for attendees to choose to present to legislators: Support Rare Disease Appropriations Priorities, Cosponsor the Better Empowerment to Enhance Framework and Improve Treatments (BENEFIT) Act, H.R 1092 and S. 526, ask Members of Congress to join the Rare Disease Caucus, and join a congressional sign-on letter to the FDA requesting the formation of an internal FDA task force to review and inform agency-wide rare disease activities.

In these meetings, I mentioned that each of these would help benefit the rare disease community, but my ask was for a proclamation officially recognizing June 9 as International Batten Disease Awareness Day. A few months later, thanks to Fogle and U.S. Congressman Aaron Bean (FL), Rep. Bean introduced a

resolution recognizing every June 9 as Batten Disease Awareness Day.

In each of my meetings, I focused on sharing family stories from within the Batten community to give the staffers a snapshot of what living with a rare disease is like.

Not long after I started this job, I was told that a big reason why they call a rare disease a rare disease is that not enough people know about it. That sticks with me each day and emphasizes the importance of sharing stories from the community.

Attending Rare Disease Week and advocating with legislative staff members are the two most empowering things I've ever done, but the fight doesn't stop there. Persistence must follow. Those on Capitol Hill need to continue to hear from our community.

While serving as a panelist during Rare Disease Day at NIH, King said, "Tell those stories – it's the most important thing you can do, and everything else will grow from there."

Everyone in the Batten community has a story to tell, and when you're ready to share it, know there are people who want to help you and listen.

Those in the Batten community share a lot of the same frustrations as others in the rare disease community, and as cliché as it may sound, there are reasons to remain optimistic. A resolution was created and helpful resources for rare disease families were presented during Rare Disease Week, including a Rare Disease Alert System and RAR-e SOURCE. The latter aims to make it easier and more efficient to access and use available data to discover and develop treatments for rare diseases. And the FDA Rare Disease Hub was announced in July 2024.

Lastly, BDSRA surpassed its original fundraising goal for International Batten Disease Awareness Day by over \$40,000 through the help of 817 total donations, 365 participants, and 35 fundraising teams.

Raising awareness, like getting a proclamation to officially recognize International Batten Disease Awareness Day, can help BDSRA and the community take a step forward to educate others by advocating for our families about the disease. In turn, this will help raise more donations for BDSRA and research, more treatments, and one day – a cure – so that we may celebrate progress and a new milestone. Together, we are Batten Advocates for a Cure.



Restricted Gifts

\$1,113

International Batten Disease Awareness Day Fundraising

\$52,824

International Batten Disease Awareness Day Participants

365

Advocacy Initiatives

10

Illuminator Donations

\$763

New Illuminator Subscriptions

99



NEW FUNDRAISING STRATEGIES PROVIDE SPARK TO BDSRA'S DONOR GOALS

It began with a \$10,000 fundraising goal.

The 2023 International Batten Disease Awareness Day fundraising campaign was BDSRA's first time using our database Charity Engine's peer-to-peer functionality. This new feature allowed Batten community members to create their own individual fundraising pages and team pages and set fundraising goals.

All proceeds would go to BDSRA, and others were encouraged to participate in more activities to raise awareness for Batten disease – such as the T-shirt design contest and the virtual 5K walk/run.

“The event has historically brought together smaller communities of people who were actively caring for or dealing with the disease, and seeing all the people come together helped them feel less isolated,” Noah Siedman, BDSRA's Database Manager, told Charity Engine in an interview.

Daily morning updates from Noah in the BDSRA staff text message group chat with a screenshot of the fundraising progress followed.

“13,000 and counting,” Siedman texted the staff on May 8, 2023.

“Closing in on 16,000 to start the day,” Siedman texted the morning of May 10, 2023.

“I should be asleep, but we got to \$19,000 in the latest few hours,” Siedman texted the group at 11:16 p.m. EDT the next day.

On May 29, 2023, Siedman texted, “40,000 here we come!”

The end result was \$52,824 raised through 817 total donations. There were 365 participants, 35 teams, a donor from each of the 50 U.S. states, and 170 first-time BDSRA donors. There were also 19 submissions for the T-shirt design contest and social media feeds flooded with Batten disease awareness.

“This was the first year we set a goal and made a concerted effort to promote fundraising for BDSRA on International Batten Disease Awareness Day. The community walked right past each goal we set!” BDSRA President & CEO Amy Fenton Parker said. “We were thrilled to support each community's twist on the day, as well, and some were really creative.”

This campaign's success and high participation expedited BDSRA's efforts to explore different fundraising avenues so the community can help the organization fundraise year-round. Those efforts led to the creation and launch of \$5 Fridays and Fam Funds.

The Fam Funds program aims to empower families to share their Batten journeys, advocate for Batten



Greatest Need

\$264,568

5 fast things you can do:

DONATE

\$36,856

IRA Rollovers, Donor Advised Funds, & Trusts

ADVOCATE

\$57,992

Bike Events, Bowl-A-Thons, & Batten Disease Awareness Day

GIFT

\$93,743

Anniversaries, Weddings, Birthdays, & Tributes

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.



disease, and raise funds for BDSRA year-round. Families may choose where donations are directed: Greatest Need, Support, Research, or Advocacy.

The Beedle family – consisting of parents Adam and Amanda, and their CLN2-affected daughters, Annabelle and Abigail – was the first family to participate in the program.

“Very early on after our children’s Batten diagnosis, we felt convicted to share our family’s story in hopes that something positive could come from something so dark and so devastating,” Amanda Beedle said. “As a family, we’ve defined it as our mission to bring brighter days to the Batten community.”

BDSRA’s \$5 Fridays program encourages Batten advocates to regularly give \$5 on Friday – weekly, monthly, or quarterly. The proceeds benefit BDSRA’s operations, such as staff, Charity Engine, office rent, and much more. Ensuring the stability of the organization means a greater impact on the community.

Many Batten Advocates explored their own creativity for funding our work. The Ohio Farm Bureau Federation of Hocking County raised \$1,252 through a tractor drive, car show, and pancake breakfast, in memory of Westley McKinley and in honor of Kyndel Roop – two local Batten patients. Westley passed away on October 4, 2022, at 16 years old. Kyndel followed him on November 8, 2023, a few months after the check presentation.

Carley and Dylan Fogle raised \$425 for BDSRA through a lemonade stand they held during the Fort Lauderdale Air Show. The duo’s efforts were in honor of their cousin, Kourtney Harmon, who passed away from CLN1 Batten disease in 2010. They encouraged thirsty passersby to buy a cup or donate online while advocating for Batten disease.

From galas to golf outings, tractor drives, and lemonade stands, financial support for BDSRA is critical to the organization and the Batten community in the fight for treatments and cures.



BOARD CHAIR ADDRESS TO COMMUNITY

Dear Batten Community,

We have come to the close of another year on our journey with Batten disease. You may be a newly diagnosed family, a family in the middle of the long and rugged journey through the disease, a family who has recently lost a loved one, or a family who continues to share the memories of their loved one's life. We are all at a different place in our journey with Batten disease, but I want to let you know that once your loved one is known to BDSRA, they will never be forgotten.

When a family loses someone they love to Batten disease, they may not be thinking clearly. I know. We lost our three girls to Batten disease, but from the first Annual Family Conference Randy and I attended through today, we have attended Sunday's memorial service. We wanted to experience what the full conference had to offer. We decided from the beginning that we were there to support families experiencing loss because we knew that one day it would be us. We visited the memorial wall and watched as families lit candles to honor their loved one(s) at the service. From there, I found what I wanted to do: make sure that every child is honored each year, never to be forgotten.

The memorial wall changed over time as more individuals lost their battle with Batten disease. We knew the memorial needed to be everlasting. That everlasting change was realized in 2019 at the Annual Family Conference in Denver when we retired the beautiful plexiglass memorial wall for a new interactive memorial that would live on forever with the stained-glass picture in the center.

When we created this living memorial for our loved ones, the cost was approximately \$85,000. We raised about \$35,000, and the board voted to pay the balance out of savings. Although the memorial is now in two containers rather than six, shipping is still expensive. We carry insurance on the memorial, and we pay for its storage as well as the storage of the data holding the memories. The cost of keeping the memorial at the conference and on our website is one of those necessary hidden costs, totaling more than \$10,000 annually.

The memorial is perfect for keeping the memory of your loved one alive eternally. Anyone can visit memorial.bdsrafoundation.org/search to remember others' loved ones and to add your own to the website, which will also live on the Annual Family Conference memorial. You can create a beautiful memorial by adding pictures, videos, letters, and newspaper articles. This tribute lives there for the world to see all year long, not just at the Annual Family Conference. There are only 652 people remembered in the living memorial, and I know there are so many more not represented. Please, take advantage of this living memorial. I have created a page for each of our three girls, and I invite you to look through our fond memories with them.

This year when you receive the Impact Report, I am hopeful you will donate to the BDSRA Foundation for its growth to meet the needs of our Batten community – but also for the living remembrance of the loved ones we have lost to Batten disease.

Thank you and may God bless you and your family,



Darlene Royalty,
Chair of the BDSRA Board of Directors



OUR BATTEN ANGELS



Jaxson James Scott O'Brien | December 8, 2012 – January 26, 2023
Josie Gail Bayer | October 18, 2011 – February 8, 2023
Johannes Nienhaus | May 18, 2016 – March 10, 2023
Max Reid | September 14, 2007 – March 12, 2023
Brody Bradley Koslowski | July 30, 2015 – March 25, 2023
Ella Dawn Nakvinda | March 8, 2019 – March 27, 2023
Samuel Russell Engman | September 24, 2011 – April 25, 2023
Lucia "Lulu" J. Guerra | July 14, 2012 – May 6, 2023
Jeremiah Joseph Rivers | June 1, 1997 – May 30, 2023
Lionard Alfonso | April 9, 1997 – June 1, 2023
Olivia Claire Burtwistle | August 31, 2003 – June 9, 2023
Seda Ormankian | Aug. 28th, 1999 – June 11, 2023
Ashlyn Marie Turner | June 28, 2023
Elif Akar | July 14, 2006 – July 1, 2023
Alexander Valance "Alex" Bryant | April 5, 1999 – July 17, 2023
Cemrenur Kisa | February 10, 1996 – August 8, 2023
Muhammet Talha Bilgiç | July 10, 2009 – August, 24 2023
Tessa Ann Bruner | September 5, 2015 – September 22, 2023
Peyton Allen Bailey Mayes | November 18, 1998 – September 26, 2023
Brock Richard Scarpetta | April 9, 2007 – October 10, 2023
Nicole Thomasena Rich | March 1, 2012 – October 23, 2023
Nataly Paradellas | March 30, 2009 – October 29, 2023
Kyndel Dawn Roop | March 31, 2015 – November 8, 2023
Jaxom Tyler Mason | June 26, 1987 – December 1, 2023





Batten Disease Support + Research + Advocacy

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BDSRAfoundation.org



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