

IMPACT REPORT

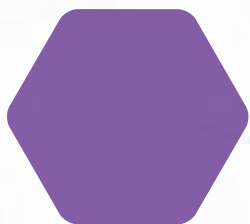
2021



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Dear Batten Community,

In preparing this Impact Report for the year of 2021, I realized that we were all emerging from significant changes in our lives. The pandemic had kept us at home for a year and we slowly began a new chapter as reopening began. We were cautious, but we learned how to co-exist with the pandemic by resuming some of our usual daily activities while adjusting to permanent changes. My first year at BDSRA was much the same.



We pulled out the 2018 Strategic Plan and used it as a road map for our work. The first paragraph directs that the plan was to inspire key stakeholders to “reimagine what BDSRA can and should be in a new era of research, drug development, legislation, and connectivity.” The board placed their trust in me, and the executive committee met with me weekly to ensure that I was on the right track. I began my virtual listening tour, meeting with families, donors, industry partners, clinicians, researchers, and our international partners. Our Zoom subscription was a great investment!

The plan affirmed the need for “increased staff and board capacity to serve our community well and an accompanying need for increased fundraising to build the organization’s capacity.” I went to work on our org chart and job descriptions for the positions needed to take us where we need to go. We needed to get the house in order, be transparent in all things, fiscally responsible, and ensure that the staff had professional development and performance reviews for guidance. BDSRA was emerging with opportunities in its complex ecosystem.

The strategic plan suggested that we needed “a menu of support options from which Batten families can choose,” and we offered virtual programming every month of the year, including the Annual Family conference. Whether it was grief support, family parties, or Ask-An-Expert, BDSRA worked to build community and offer a variety of programs. Our families gave equipment to each other, received grants for joy, and others got support in their greatest time of need. BDSRA conducted a soon-to-be-published family needs assessment, as suggested in the Strategic Plan, and it was completed by year-end.

Research was one of the biggest victims of the pandemic. Labs were constricted from lack of PPE, complicated cleaning processes, and inability to perform their studies safely. BDSRA grants were extended—some even into 2022. There was no need for a granting cycle in 2021 because it wasn’t happening.

However, in listening to our community, it was clear that a Science Officer (of sorts) was desired by many and that there would be support from some to help with the search and salary. First, we needed to create savings for research grants to begin again, but the position was created, and the role emerged from a goal to a reality.

The board's Advocacy Committee was a product of the Strategic Plan, and this pillar of BDSRA really hit its stride as the year progressed. BDSRA staff and volunteers met with legislative representatives on virtual Hill Day, participated in an industry partner's video for Rare Week, and advocated for Newborn Screening initiatives across the United States. International Batten Disease Awareness Day was celebrated by many, and we partnered with our international alliances to spread the word worldwide. The Illuminator went out every month, and social media really took off with the addition of a Marketing & PR Coordinator on staff.

When I began with BDSRA in January of 2021, I committed to lead the organization while rooted in its Mission, Vision, and Core Values. I was charged with leading a family-centered organization committed to collaborative research, and acting as good-faith partners in the pursuit of excellence and legal adherence in nonprofit stewardship and management. In the pages that follow, I hope you will find the fruits of our work emerged from the plan set before us and have prepared us well to work toward our mission – a world without Batten.

With Hope,



Amy Fenton Parker
President & CEO



BRINGING JOY ON THE JOURNEY

Joyful moments can be few and far between for families journeying the rigorous and emotional road that is Batten Disease.

That road may include financial challenges and equipment needs. Through its support programs, BDSRA helps aid these difficulties with the Batten Family Help Grant – a \$1,700 grant program to be used for emergency travel, excessive hospital bills, unexpected costs, etc., and the BDSRA Equipment Exchange – a program for individuals and families to gift unused equipment to other families in need.

The moments that bring smiles to the faces of Batten patients are the ones that families cherish most and that last a lifetime. Some of those moments are made possible through a collaboration between the BDSRA and ForeBatten Foundation, called Fore the Journey.

Fore the Journey is a grant program that offers experiences, gifts, or trips for Batten patients and their families with hopes of contributing those happy moments.

In 2021, Fore the Journey gave out 18 grants towards providing joy for families in their Batten journeys. From fence repairs to gifts such as a trampoline, karate classes, patio shade, and an adaptive bicycle, these joyful experiences can be as unique as the individuals requesting them.

For Susan Stilwell, the mother and full-time caregiver of Harry, whose blindness, mobility issues, and cognitive delays from Batten limit his participation in certain activities, Fore the Journey made it possible for him to have one-on-one karate lessons with a special needs sensei twice a week.

“He absolutely loves it and talks about being a ninja every day!” Susan said. “As Harry’s full-time caregiver, I don’t earn an income so without Fore the Journey this opportunity would have been out of our reach. **“This program has brought a lot of joy into Harry’s life, and we are extremely grateful!”**”

SUPPORT

Restricted Gifts

\$18,605

Batten Family
Help Grants

5

Equipment
Exchange Grants

4

Fore the Journey
Grants

18

Virtual Programs
Provided

19

New Family
Engagements

69

Teach & Be Taught
Curriculum
Distributed

7

The Fore the Journey grant also helped bring joy to Sabrina Castillote's son, Malachi, in playing music. As Malachi's vision has deteriorated, it became more difficult for him to play the 1920s box piano that was in their home.

Sabrina took Malachi to Guitar Center several times to play a digital piano that lit up when it is played. The grant allowed the family to bring home that same digital piano which accommodates his declining vision.

"He would play this same piano every time till I would say it's time to go home," Sabrina said. "Malachi has played for years by ear and having a piano that lit up so he can see the keys has brought this boy so much joy in such a hard time in his life."

And a trip to Disney made Christmas Day even more magical for Marlo Schinell's son, Dante, and their family.

"My entire family and I extend extreme gratitude to ForeBatten and BDSRA for granting the most magical Christmas wish," Marlo said. "Our hearts are still full from the experience."

These are just a few examples of the positive impact the Fore the Journey grant has had on Batten families on their Batten journeys last year.

While each Batten journey has its ups, downs, and unknowns, BDSRA strives to create partnerships and programs that support families battling through Batten.

Each of BDSRA's support programs aim to provide patients and their families with financial, resourceful, and emotional relief through their Batten journeys. No matter where patients and their families are in their journey, BDSRA is proud to support everyone affected by Batten and be a source of light on an uncharted road.

COLLABORATING ON NCL CONFERENCE

Research is a crucial pillar of BDSRA's mission for a world without Batten Disease.

Part of that mission involves providing a forum to present the latest advances in scientific, clinical, and translational research into the NCLs and making sure all of that information is accessible to Batten families. Through steadfast partnership and care, the scientific community behind BDSRA continues their commitment to excellence in their work and to better the lives of Batten patients and caregivers.

Those were among the main objectives during the 2021 NCL Conference held at Washington University in St. Louis this past October, led by Dr. Jonathan Cooper. Among the other main objectives included selecting presentations on scientific merit from submitted abstracts, rather than inviting established speakers, and promoting the highest standard of scientific and clinical presentations, while still making this science accessible to affected families and other individuals not directly associated within the scientific field.

BDSRA President and CEO Amy Fenton Parker and Vice President of Support & Advocacy Morgan DeBoth served as Co-Chairs for the family portion of the conference. BDSRA also helped throughout the planning process and served as the Fiscal Sponsor for this year's conference.

The conference was held a year later than originally planned due to the COVID-19 pandemic. The NCL Conference is held every two years with the location alternating between the U.S. and Europe.

"The International Conference on Neuronal Ceroid Lipofuscinosis (NCLs or Batten Disease) is the only major international scientific meeting that focuses on advances in scientific, clinical, and translational research into these life-limiting inherited pediatric disorders," Parker said during the welcome address Day One.

The conference included multiple science presentations, poster sessions, family decoding sessions, and research update events. Both virtual and in-person attendees were able to ask questions during the Live Q&As following each respective session.

Genetics and cell biology, disease mechanisms, new technologies, and preclinical translational work were among the topics discussed and decoded for the near 100 people in attendance at the hybrid conference in St. Louis and 200 who joined virtually.

“The NCL Conference has been a great place for us to see the scientists that we have funded over the past 10 plus years through Hope4Bridget and discuss future projects that can get us to our end goal -- the cure for Late Infantile NCL,” David Kennicott said. **“We have learned so much from these scientific sessions and conversations with the scientists over the years and we have been able to provide them with real life experiences about living with CLN2 with the hope it will help develop future research projects.”**

Among those who attended the conference virtually were 26 family participants who attended at no cost thanks to an anonymous donor.

Even with the 2021 NCL Conference being a hybrid one, it brought the Batten family and research communities together to not only share new information, but also help those affected by Batten to understand and apply that information in each of their own journeys.

As research continues, BDSRA will continue to help provide opportunities for our Batten families to share their lived experience and work towards achieving our mission of a world without Batten disease.



RESEARCH

Restricted Gifts

\$63,677

Research Grants **\$40K**

Ask-An-Expert Research Edition **5**

Research Conferences Attended **14**

Researchers who Spoke at Batten Sponsored Events **51**





AMPLIFYING FAMILIES' VOICES

No matter where families are in their Batten journeys, BDSRA is here to amplify families' voices and make them feel heard. Advocacy is key to spreading awareness and crafting a better tomorrow for patients and families.

BDSRA's advocacy efforts range from supporting family fundraisers and advocating in schools, to supporting local, state, and federal legislation that would benefit those affected by Batten and other rare diseases.

One of those family fundraising collaborations is Bike for Batten, inspired by Bekah Murphy's Batten-affected nephews Titus and Ely. Titus passed away at the age of six, and Ely has become living proof of the progress made in Batten research thanks to enzyme replacement therapy. All donations from the Bike for Batten fundraiser were set aside in a fund specifically to support future research initiatives.

"We are forever grateful for the work being done," Murphy said. "The research teams and doctors are currently in the midst of finding a treatment to prevent blindness in children as well as gene therapy. There is a lot more work to be done and it is exciting!"

International Batten Disease Awareness Day, Rare Disease Week, and RUSP alignment legislation are also among the BDSRA's advocacy efforts to achieve its mission for a world without Batten disease while continuing to support Batten families. Our organization strives to meet families where they are to empower them. From running for school board to leading a fishing tournament, and educating friends, BDSRA is committed to sharing the stories of patients and families.

Among those families is the Beedle family, who has two CLN2 affected girls – Annabelle and Abigail. Annabelle's diagnosis is what led to her parents seeking testing for her younger sister Abigail. Abigail was the youngest U.S. patient to receive Brineura, the only FDA-approved treatment for CLN2 Batten Disease, at only 10 months old.

The Beedle family first reached out to BDSRA in February of 2021 and the relationship between the family and organization only grew from there along with the family's willingness to allow BDSRA to advocate for them. This included an invitation for BDSRA to attend the Beedles' fundraising event last fall to advocate. During that event, BDSRA was able to set up a table and provide educational materials to their local community on Batten disease.

Advocating for Newborn Screening has been a large push for BDSRA in 2021. Morgan DeBoth, Vice President of Support & Advocacy, serves on the Community Advisory Board for ScreenPlus – a comprehensive, flexible, multi-disorder newborn screening program in New York State. Stories such as Abigail's allow us to tell the impact of newborn screening initiatives and the importance of early access to genetic counseling for our community. Sharing real families' stories helps us make progress in states like Arizona and Ohio, who in 2021 passed RUSP Alignment legislation.

"Our children have a voice," Amanda Beedle said, the mother of Annabelle and Abigail. "Advocacy is vital in allowing their stories to be heard." Those stories are critical to spreading awareness and leading initiatives for positive change for the Batten and Rare Disease communities. BDSRA strives to make these voices heard by joining patient advocacy groups and Rare Disease Legislative Advocates to achieve better support and services for Batten families.



Restricted Gifts

\$5,000

Family Needs Assessment

Completed in Alignment with
the 2018 Strategic Plan

Toolkit Tuesday

Launched Rare Disease
Week

Staff Speaking
Engagements

4

States Added
RUSP Alignment

2

International
Batten
Awareness Day
Participants

229

LOOKING OUT FOR FUTURE FAMILIES

Donating to the Batten Disease Support and Research Association this past year was a decision that made sense to Warren and Brenda Pfohl.

The couple and co-founders of David's Refuge, a New York State-based non-profit that provides respite, resources, and support to parents and caregivers who have children with special needs or a fatal disease, wanted to continue to help families affected by Batten disease and give back to the organization that's been with them every step of the way during their Batten journey.

Their son, David, battled Batten disease for 13 years before passing away in 2009 at the age of 20.

"They didn't even know where it was on the DNA strand where the things were broken down (when David was diagnosed), so we've made a lot of progress, but we have so far to go," Warren said. "And so we want to just continue to be a part of one day beating this horrible, horrible thing."

"They were there in a moment of complete terror and horror and brokenness," Warren added of BDSRA. "Anytime we had questions, we had a resource to be able to go back to. They were the only organization doing something that was attempting to find a cure for what we were told was an untreatable, fatal progressive disease, which it was."

When it came to giving back to BDSRA, Warren wanted to do so in the most strategic and effective way possible – a stock gift. A stock gift is appreciated stock that is donated by a seller who then doesn't have to pay taxes on it if there is a capital gain on the stock – maximizing the amount donated to BDSRA.

"BDSRA, for us, was there when our son David was diagnosed and kind of journeyed with us through his passing, and I want every dollar that I have to go to them and I'm a believer in paying my fair share in taxes," Warren said. "But if I have a choice between giving \$35,000 to the government or \$35,000 to BDSRA, to me, it's a no-brainer."



Warren says it's a win-win for both parties involved – the organization receives a valuable donation, the giver avoids capital gain and the total amount donated is tax deductible. The stock gift is also a perfect example of the unique and various ways people can donate money.

“We do most of our giving through appreciated stock and we also use a donor advised fund, which is another great tool for people to consider when they're looking at making investments or contributions to nonprofits,” Warren said.

“What also is helpful is, for example, a lot of times if I donate stock, what I will do then is I will repurchase that same amount of stock and then my investor is able to rebalance my portfolio. So that's a great way at that point in time for them to kind of look at how they might kind of change some of your investment strategy.”

For the Pfohls, the donation goes a long way towards helping families who are still on their journey of that original diagnosis and facing the road of uncertainty that is Batten disease. It's another way of showing their appreciation for BDSRA and continuing to do their part in helping the organization fulfill its mission and vision.

“They were just there for us,” Warren said.
“And for that we are eternally grateful.”



Unrestricted Funds:

\$724,108

5 fast things you can do:

DONATE

Make it possible to fund our mission, donate today.

ADVOCATE

Join a core group of supporters to be an advocate.

GIFT

Make your next occasion an opportunity to gift to Batten Families.

EMPLOYER MATCH

Ask your employer about matching your charitable donation.

SHARE

Share your story to raise awareness for Batten Disease.

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



BOARD CHAIR ADDRESS TO COMMUNITY

Dear Families:

I have been proud to serve BDSRA on behalf of our community for some time. I became Board Chair at the beginning of the pandemic in 2020—and learned to manage both—at the same time. A lot has changed since then, and we are a stronger organization and community for it.

As your Chair, I have seen first-hand the need to promote, expand, and improve BDSRA for the future. Last year was a year of change and growth. Our board is working very hard to support our staff, work with them to be open to the needs of our community now, and plan for what our community needs and wants in the future.

We cannot go back to life before COVID, but as we learned last year, we can transition to this new way of life and work together toward our shared mission and long-term vision: a world without Batten.

God bless our families, and I hope to see you at the 2022 Annual Family Conference in Cleveland.

A handwritten signature in black ink that reads "Darlene Royalty". The signature is written in a cursive, flowing style.

Darlene Royalty,
Chair of the Board of Directors





BDSRAfoundation.org



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