



**BDSRA ANNIVERSARY**  
**1987-2017**

**BATTEN DISEASE**<sup>SM</sup>  
Support and Research Association

**2017**  
Annual  
Report



# Letter from BDSRA's Executive Director

One of the best parts of my job is to tell BDSRA's wonderful story at speaking engagements here and abroad. It is always an uplifting experience to answer questions about how we help families, engage with pharma and biotech partners, and focus on the future to end Batten disease. This week, to prepare for a talk at a national research conference, the sponsors asked for a paragraph description of BDSRA:

*For 30 years and with great pride, BDSRA has served families of children with all forms of Batten disease—of which there are 14. A full complement of support services is available to families including a large family conference held each summer, a vibrant online community for support and education, grants for equipment needs and more. BDSRA's research program has funded registry, animal model, biospecimen collection, and other major building blocks important to crosscutting Batten scientific and drug development progress. With many others in rare disease, we advocate for a brighter future for all, by building coalitions, educating decision makers, and representing the important patient voice whenever possible.*

When I read this statement again and reflected on it, I'm filled with pride and say 'They did this. They kept the faith. They made this difference in the world. The 'They' is You. You have been part of this vast network of caring and believing for so many years. Many of you have experienced the ultimate sacrifice of losing your children to Batten and yet, continue to contribute financially and with your volunteer time to make it better for others.

From the BDSRA's Board of Directors, staff, and our research and clinical partners, we acknowledge and thank you. Enjoy reading about this community that thought it could—and has, for 30 years.

In hope,



Margie Frazier





## Welcoming Newly Diagnosed Families to Our Community

BDSRA, founded on the core value of supporting families impacted by Batten, carries out that vision each day. Meeting families wherever they are in their journey, whatever form their loved one has, and at whatever level of engagement they find personally valuable, we are there.

For many families, their relationship with BDSRA begins immediately after diagnosis. Families may come to us from their physicians, social workers or genetic counselors. Many discover us online searching for information on Batten disease. Others reach out to another Batten family who then connects them with us for further support. Whatever the route, our phones, emails and office doors in Columbus, Ohio are open.

During early conversations with a newly diagnosed family, we learn about their child or children (affected and unaffected), other family members, and friends. We learn about each family's support network to best connect them to our services and services they may be able to receive locally. Most importantly, we want each family to know that we exist as an organization dedicated to advocating for their needs. We are actively building a better future for children like their own.

*"When we found BDSRA it was like the weight of the world was off our shoulders because there were other parents in the same situation as us. Over the years we have found community and family to share our journey. We are not alone." – Darlene Royalty, Bereaved Batten Mom from Ursa, Illinois*

As part of an effort to support the growing number of newly diagnosed families in the U.S. and beyond, BDSRA partnered with parents and physicians to build an educational piece to give families a condensed but comprehensive view as quickly after diagnosis as possible. The "New Family Packet" is tailored to the specific form of Batten disease of which the child has been diagnosed and contains information on genetic inheritance, progression, and the ways BDSRA can support.

We want all families who receive this diagnosis to have access to the wealth of knowledge accumulated over 30 years of families' experiences. To ensure that as many new families as possible receive this introductory resource packet, they are available for download on our website. Additionally, we mail them to families we have direct contact with, and we also send bundles of packets to Centers of Excellence, physicians, genetic counselors, and other clinicians we meet at conferences throughout the year. We have received feedback that these experts are grateful to have them on hand so they can fully support a new family they diagnose. While our wish is never to have to welcome another new family to the BDSRA community, we know it is essential to be there at this early time in the journey and will continue to evolve these educational materials.

For more than 30 years, we have grown to welcome many new families year after year. Our collective network of experience is enormous. Clinicians and industry professionals will often say that families are the real experts on Batten disease because no one can give you more valuable information than the patient community. BDSRA works year-round to maintain the best knowledge and relationships possible to provide families with what they need when they need it.



# Families Build the Foundation: Then and Today

A Batten disease diagnosis didn't always come with an introduction to a community of people who had received the same life-altering news. Before the internet, before most physicians knew what it was, and before the BDSRA was officially formed, a small group of dedicated parents worked to create a support network for families. Evenings would be spent sitting around the kitchen table making phone calls and reading any information they could find.

In 1987, The Batten Disease Support and Research Association (BDSRA), was officially incorporated as a 501(c)3 nonprofit organization. The founding families had the hurdles that newly diagnosed families face today along with very little to no access to further information or access to experts such as physicians, clinicians, teachers, or researchers. These families worked to organize regional meetups, barbeques and social gatherings bringing small groups of families together to talk about the care of their children and fundraise to grow the capabilities of the network.

The first family conference was in Kerner, LA in 1989, just two years after BDSRA was formed. It was a game-changer for families across the country that could now make connections with other families, see children like their own, and truly know and feel that they were not alone. An entire group of passionate parents were going through the same things and fighting for the same future.

Today, the same is true about the importance of a network of families and the annual conference. Many families head home from the conference with the renewed energy to make it through the next year with a child with Batten disease.

We have 4 full-time staff members, 11 members on our Board of Directors, an incredible community on Facebook that has grown into a global family network, and an information highway between BDSRA and physicians, scientists, families, and caregivers of many kinds.

The future for the Batten community has never been so full of hope. Every day we think about the families who have paved the way for us to be here and continue to support us so selflessly. To all of those family members whose children are no longer with us, please know you are always on our minds, and we are so grateful for what you have given us. We continue to share this journey together toward a brighter future.

A special  
thank you  
to these very early families:

**The Cahill Family**  
**The Davis Family**  
**The Franklin Family**  
**The Grant Family**  
**The Johnston Family**  
**The Joyce Family**  
**The Killen Family**  
**The Klee Family**  
**The Luce & Reese Family**  
**The Milani Family**  
**The Skippon Family**  
**The Walsdorf Family**  
**The Wheeler & Ciaccio Family**





## Support Programs Evolution

Supporting families is a core element in BDSRA's mission. At the heart of our support programs is the knowledge that connecting with others who are sharing a similar experience can lift some of the burden.

It was with that in mind that the BDSRA Annual Family Conference was created nearly 30 years ago. Consistent support from families and funders has allowed us to develop opportunities for families to connect with each other and expert sessions on topics like symptom management, education issues, caregiving, respite, grief, and others. Families tell us these resources are paramount to navigating their life with a loved one affected by with Batten disease. The family conference also provides attendees updates from researchers, interactive childcare for affected loved ones, and time for families to meet with physicians and ask them questions.

Another way BDSRA facilitates peer support is through the SIBS Program. The BDSRA SIBS program was started by siblings for siblings and is one-of-a-kind in the rare disease community. It continues to be run by a group of dedicated adult siblings and grows each year. We know that there is no one who understands the unique experience of having a sibling with Batten disease better than another sib.

Acquiring the specialized medical equipment a child with Batten needs can be a long and involved process, one that not always fully covered by insurance. Our equipment exchange program was developed as a way for Batten families to support one another through donating their gently used medical equipment to families in need. The program has evolved from a storage facility run by BDSRA to a shipping reimbursement system to ensure quick delivery of items to the recipient. Parents find it to be a great way to continue their child's legacy.

Schools are an important socializing and learning environment for Batten kids. To help Batten children be supported in school BDSRA sends Teach & Be Taught materials to hundreds of families, teachers, and school officials. In addition to ensuring the materials are updated with our growing knowledge and in the hands of anyone who needs it, BDSRA has flown out education consultants to meet with families and teachers across the U.S.

BDSRA's support programs are ever-evolving based on feedback from parents and family members. The participation of our dedicated families in peer-support programs facilitated by BDSRA have made our community an example for other organizations in the rare disease community.



# Growing Physician Awareness

Just as families need support, so do physicians. BDSRA has steadily developed relationships with experts across the nation. Several Centers of Excellence originated, having seen a growing group of Batten patients over time.



The breadth and depth of physicians who see patients with Batten disease has grown exponentially since the start of BDSRA. We are grateful for teams like the one at the Children's Hospital of Orange County (CHOC), led by Dr. Ray Wang that take a special care of our Batten families. He is currently treating kids with CLN2 with the first drug for Batten disease, Brineura™. Dr. Wang has served families with the CLN1, CLN3, CLN6 and CLN14 forms of Batten and cares for patients with other lysosomal conditions. The CHOC team exemplifies the complex care needed by patients with Batten disease. We are excited to share their story with you.

Dr. Wang at the CHOC was a pediatric resident in his 2nd or 3rd year when he first saw a Batten patient. During a typical well-checkup, a mother shared that her daughter started bumping into things and was having to sit closer and closer to the television. This experience sparked his awareness and knowledge of Batten disease.

Over time, he began to see other Batten patients. The James Family (parents Beau and Suzette and daughter Maya) talked to Dr. Wang about the work we were doing at BDSRA and suggested he connect with us at the upcoming WORLD Symposium for Lysosomal Storage Disorders in San Diego. At that time, Dr. Wang and Margie Frazier met at WORLD there was no Brineura treatment at CHOC and no green light that they would be part of the early access program. Today, Dr. Wang sees approximately 15 patients with Batten disease. Because CHOC was an early adopter of the early access program for Brineura, other hospitals have sought out advice on how to start up an infusion system. We are so grateful for physicians like Dr. Wang and teams like CHOC who are willing to take the time to share the expertise they have learned so others don't have to reinvent the wheel trying to accomplish something new for their hospital.

The WORLD Symposium where CHOC and BDSRA connected has grown from around 200 people to more than 1,000 in recent years. It is a very dedicated field of professionals. Many of which attend the BDSRA Family Conference to speak with families from across the country. We look forward to seeing Dr. Wang and more Batten physicians at our 2018 conference in Nashville, TN.

"This program has been great for multiple disciplines within the hospital; we want to get research folks in the hospital. Our nurses and pharmacy invested the time to figure out how we would pull it off, spending time rounding up the infusion kits, so neurosurgeons are ready to serve patients in a timely way on infusion day," said Dr. Wang.

"It has been a fun ride, really rewarding, and we are hopeful for the future. We all recognize that an enzyme is not a cure and we have to keep working hard for something to halt or reverse the progression itself. This enzyme is for one form, there are more kids that need to be taken care of. It is a good beginning and I look forward to helping more."

BDSRA continues to improve the patient experience by filling information gaps, connecting families and physicians to one another and growing the network of physicians that talk to each other. This information highway is ever-changing. Professionals like nurses, physical therapists and genetic counselors are part of the growing group of clinicians who communicate with us and each other. We are committed to facilitating an even more rich environment of experts for families to rely on. With your support and communication that network keeps growing.

# The First-Ever FDA Approved Treatment for Batten Disease: Brineura

Before the clinical trial and before the naming process, the BMN190 molecule was a seed in a project that many hoped would grow into a meaningful treatment for Batten disease. As families and researchers in the rare disease community know, seeds of promise do not always blossom into what we hope they will become. The long and complex road to approved treatment may not yield tangible benefit for the patient, among many other potential complications. However, in 2017 our community received a big win. Brineura became the first FDA approved treatment for Batten disease, which is a monumental stride in our efforts.

Pascale Tiger, Ph.D., Scientist II, Protein Biochemistry at BioMarin met Tracy VanHoutan in 2009 at a scientific conference, about two years after she had begun research in CLN2. It was during that interaction that Tracy put his son, Noah, on Pascale's radar. This connection was a great driver of passion behind the project. Noah's story was subsequently shared with the team at BioMarin. Videos originally created for Noah's Hope were now being used to educate and rally the Brineura team to tackle something that had never been done before with break-neck speed.

## Benefits to having the patient organization involved throughout the process (shared by Tom Lester, BioMarin):



### Families

The families are the experts of the disease. They often know more than anyone else.

Learning what is important for them, what they are worried about and what they are thinking about.



### Communication

You get insights on the spectrum of the disease. Scientists will work to make broad conclusions, families can tell you what is happening with their child. For example, suctioning (on paper) seems like a low-level concern compared to the other items that the child is dealing with.

A parent who is suctioning their child hundreds of times a day can shed light on this as a big concern.



### Connection

Having a genuine connection to the faces and lives of children living with Batten disease. For example, when you are reviewing a clinical study program at 10:30 p.m. instead of sleeping, it really helps to be clear on what (and who) you are doing it for.





BioMarin began engaging families through BDSRA in 2013. Their goal was to share their thoughts surrounding a clinical trial for CLN2 enzyme replacement therapy and get the input of the families living with this disease. Seeking out input and engaging with the patient perspective through organizations like the BDSRA and the BDFA was a critical component throughout their entire drug-development process. Although they could not be sure of the outcome, BioMarin went into the process from the beginning knowing what was at stake. “We’ve got one swing at this, and a single isn’t going to do it. We’ve got to hit a home run,” shared Tom Lester, a Senior Director of Product Development at BioMarin.

Families entered the clinical trial in 2014. BDSRA was the go-to for families in the U.S. trial, helping to navigate the system, which included extensive travel or relocation, living arrangements, coordination with the hospital and local schools, and various other support along the way. Families – and just about everyone involved in the program – were walking forward into uncharted territory and BDSRA worked to fill any gaps that came up. “It was always a partnership. If families had a question for BioMarin, they usually went to BDSRA first,” said Lester.

The FDA and BioMarin both told us that without patient voices at the table in the drug development process, there would not have been an approved treatment. We would be back to the drawing board. Luckily, Batten disease has the presence of a strong patient advocacy organization, and BioMarin chose to involve the patient voice through BDSRA in every step of the process. The result was remarkable. A disease that had not a single effective treatment option for any form of the disease now has an approved treatment and the attention of the global scientific community. We know that there is more work to be done, to make Brineura the best it can be and for the other 13 forms of the disease without a treatment option.

We must take a moment to acknowledge this accomplishment. We thank the tireless effort of scientists, families, clinicians, patient organizations, and the incredible group of people who built the ladder of foundational research in Batten disease that made this trial – and every trial – possible. Thank you BioMarin for intentionally seeking out a consistent communication channel with the patient voice via BDSRA and allowing it to help you deliver an ideal outcome with meaning and hope to families. Together, we now share a significant victory and a blueprint for future efforts and we couldn’t be more grateful and proud.



## Facilitating Clinical Trials: The Role of BDSRA

We receive many calls from biotech companies that want to know if there will be a strong patient advocacy organization for Batten disease that can help facilitate the clinical trial process. They understand that the involvement of a patient advocacy organization is paramount to the success of the trial. BDSRA is proud to serve in that role for the entire Batten disease community in the United States.

We continually build these relationships, maintain communications channels and provide the best guidance possible. We fiercely adhere to ethical standards and stay true to our core value of having the best interests of families with all forms of Batten disease in mind in everything we do. Often we are asked to review materials, identify gaps and make suggestions based on the subject matter expertise from the patient voice. Because of our long-standing relationships with families, some of them have been involved in trial development. They are excited to lend their experience to help new trials and trust us to deliver their message.

### Some common ways we support include:

- Connect with patients/families, communicate, educate and request collaboration
- Help with shortening the diagnostic process
- Speak with and encourage companies and institutions to consider projects in Batten disease
- Help families navigate the trial process and work with their insurance companies
- Have staff capability to work hands-on with various needs that come up, filling in gaps to help the trial to run smoothly for patients
- Review project protocol and related materials to provide our feedback
- Support with post-trial items, i.e. Educate FDA/ Impact Regulatory Pathway

We not only ensure companies wanting to pursue a trial in Batten have every resource we can provide, but we will also collaborate where appropriate, seek out key areas for progress and amplify the patient voice in the process. We learned with Brineura that the tipping point for the FDA was seeing the disease through the eyes of the patient. When you are making a donation to BDSRA, you are doing much more than ensuring families have support; you are funding these crucial activities, essential to trial success and the development of treatments.





## Educating Peers & Influencers

Every year, we partner with other organizations, collaborate on educational materials and attend or present at conferences central to the needs of the Batten disease community. Much of this work is deeply embedded into the way BDSRA advocates for families. It is not often that we take the time to reflect on the incredible work accomplished through partnerships to influence a larger audience. It is important for key influencers to be aware of the needs of our community and engaged in supporting progress for our families.

Organizations like these provide a strong network of allies in the world of rare disease:

- The National Organization for Rare Disorders (NORD)
- Global Genes
- Rare Diseases Clinical Research Network (RDCRN)
- Rare Disease Legislative Advocates (RDLA) a program of the EveryLife Foundation

By banding together, our legislative influence is stronger. Our community still benefits from the Orphan Drug Act of 1983, which helped address the lack of treatments for small patient populations and the Individuals with Disabilities Education Act (IDEA). Each month, NORD hosts an update call for members on the latest in Federal and State issues, like the Children's Health Insurance Program (CHIP) and 21st Century Cures. Many Batten families use their voices on Annual Rare Disease Day virtually or in-person in Washington, D.C. in late February.

Our advocacy efforts extend beyond the rare disease community. The annual conferences of the Child Neurology Society, American Epilepsy Society, and National Society of Genetic Counselors are all places where you can find BDSRA engaging with professionals impacting the lives of Batten children. Physicians and clinicians attending these meetings are often the professionals diagnosing and providing ongoing care for our families. Often, they are just as excited to learn about us as we are to help educate them. We want them to know a comprehensive patient support organization exists, and they want someone to help field questions and provide information that only people with existing experience with Batten disease can provide. We give them hard-copy materials to take back to their office or hospital and maintain an open communication channel via email and phone with a network of professionals.

Speaking engagements are another way that BDSRA educates and communicates with audiences that matter. Increasing numbers of calls for BDSRA to attend events and talk about the needs of the Batten community are encouraging signs for the future. In 2017, Margie Frazier was a speaker at the National Institute of Health (NIH), the nation's medical research agency, which provides millions of dollars of funding to researchers that study Batten disease. As we look ahead, one thing we know is that we don't have to do it alone. We have an incredible network of peers and leaders that want the same thing we do, the best for Batten patients.





Doose Syndrome  
 Retinitis Pigmentosa  
 Benign Nocturnal Epilepsy  
 High Sensory Integration Disorder  
**Lennox-Gastaut**  
**Intractable Seizure**  
 Lebers Amorosis  
 Rod Cone Dystrophy  
 Bipolar  
 Schizophrenia  
 Color Deficient  
 PDD-NOS  
 Silent Seizures  
 Complex 1 Deficiency  
 Bardet-Biedl  
**Epilepsy**  
 MR  
**Infantile Spasms**  
**ADHD**  
**Autism**  
 GTP Cyclohydrolase 1 Deficiency  
 Complicated Migraine  
 LGS Learning D



ADD  
OCD  
es  
Syndrome  
Behavior Disorder  
Kargardt's Disease  
S Anxiety Disorder  
Muscular Dystrophy  
Sialidosis  
CMT ML4  
Disorder

## Progress with Earlier Diagnosis

While a Batten disease diagnosis is something no one ever wants to receive, it is crucial the family have an accurate diagnosis as quickly as possible. It can determine eligibility for a study or treatment, and it is essential to creating a care plan at home and in school to ensure the best quality of life for a loved one. Often families may not have an accurate diagnosis because their children may be treated for a long time for epilepsy or symptoms, while never being tested for a genetic root cause. The goal is clear, work toward accurate diagnoses in a shorter amount of time.

We asked Batten families to complete a survey that asked a variety of questions to identify needs and priorities of the Batten disease community. From that survey we built a needs assessment. One key finding was that it takes approximately 3-5 years for families to get a diagnosis. During that time, families were given an average of 2-3 different diagnoses before arriving at a Batten disease. The entire diagnostic journey is taxing, trip after trip to different doctors, not knowing the root of the symptoms, or what you can do to best care for your child – the timeline is too long.

Batten patients are often diagnosed by physicians ordering a genetic test called an “epilepsy panel” that includes dozens of possible diagnoses. The vast majority of these tests for genetic diseases in children are from several major companies. We have advocated with these major genetic testing companies to ensure they are covering all forms of Batten disease instead of just a few. One panel that has over 180 diagnoses involving seizures—[behindtheseizure.com](http://behindtheseizure.com), is free to physicians for families needing quick answers without insurance delays. As BDSRA staff travels to medical conferences, we help educate clinicians about the importance of genetic testing.

We are hopeful about future developments that would decrease the time to diagnosis, and increase the level of support from genetic counselors or other medical professionals for Batten families. To all the families who participated in the needs assessment, we thank you and continue to tackle the issues you tell us are important.

# Developing Building Blocks to Treatments

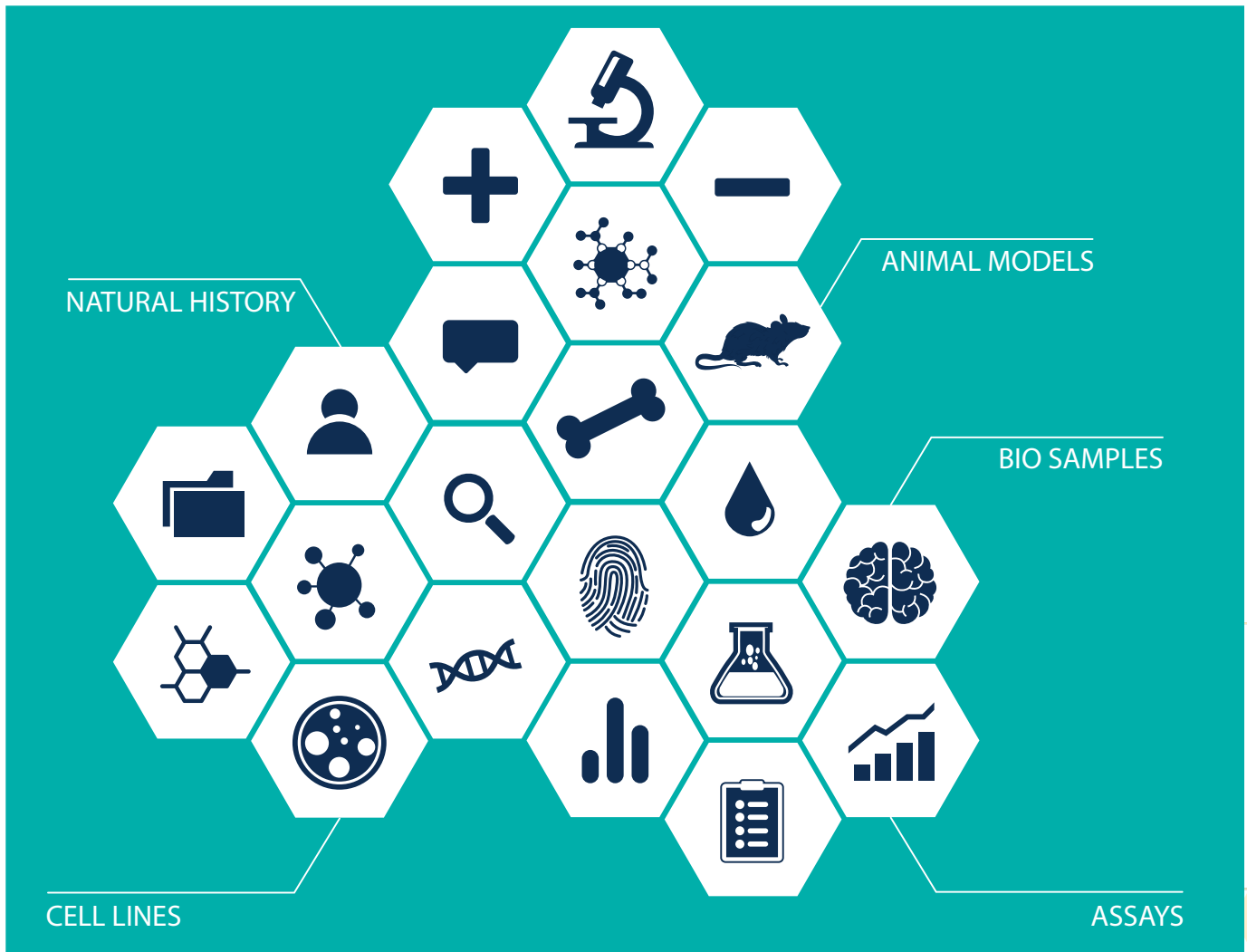
BDSRA's mission is and always will be to support families to the best of our ability, which includes funding research directly, finding ways to collaborate with the broader research community, and making scientific progress in all forms of the disease.

In turn, those researchers have been able to leverage millions more from the NIH and other sources. We are grateful for our donors who, year after year, fund foundational research tools that further Batten disease research efforts worldwide.

BDSRA donors and families have contributed financially and physically to create the building blocks required for basic and translational scientific research. These foundational pieces make the clinical trial process, treatments, and cures possible.

As research efforts expand, interest in Batten disease from biotech companies grows. Thanks to our community, we can actively connect researchers to resources, like these existing and evolving building blocks, which can be used for further research and support treatment development.

Together, over the last 30 years, we have funded 7 million dollars in grants to nearly 100 different researchers.



\* Information provided is intended to provide a high-level overview of treatment development and is not the only route that can be taken.



# Foundational Research

Getting a treatment from researchers' scientific bench to patient bedside takes years, often decades. The process starts in prediscovery studies to better understand the underlying mechanisms of the disease. Those findings are translated into preclinical studies where potential treatments are tested in the lab and in animal models. It is not until a treatment is successful in the lab that a researcher or company can apply for a clinical trial at the Food and Drug Administration (FDA).

The foundational tools we are now able to provide are essential pieces of the precovery and preclinical stages for all Batten projects. It is especially vital in rare diseases like Batten for communities to work together on these projects because the number of patients is low and the resources are harder to earn. The research community could not have developed these tools without the support and participation of Batten families and researchers across the world.

The impact of the BDSRA network that has been built through generous families supporting science is remarkable. Creating tools that speed up the precovery and preclinical stages of developing a treatment is a unique asset our community provides. We are consistently sharing information and looking for ways to connect and partner for the advancement of science. Funding building blocks that support the entire research community is central to BDSRA's mission. Anyone who partners with us is contributing to building the evolving research tools which fuel robust scientific progress with treatments and cures for Batten disease.

## BDSRA amplifies the patient voice

Throughout a treatment research and development process, BDSRA amplifies the patient voice to best inform and collaborate with scientists and other key parties. With your support we:

- Coordinate the collection of patient data through registries, biosamples, and focus groups
- Encourage research focus through personal connections at the annual family conference and clinic/lab days
- Encourage sharing of data by requiring all BDSRA grant recipients to sign on to data sharing
- Serve on government or industry advisory committees
- Advocate for regulatory changes that will facilitate rare disease clinical research
- Work with industry on trial design and patient recruitment

## 2017 Family Conference: Spotlight on Affected Kids Care

At the 2017 BDSRA Annual Family Conference, we celebrated our 30th year as an organization by honoring the families who built the conference from the ground up. One aspect that makes our conference unique is our Affected Kids Care. Care for affected kids is an integral part of our conference. It provides families freedom to attend sessions, meet with researchers or physicians, and maybe enjoy some respite time. For this part of the program to be successful, expert care must be in place for families to feel confident that their children will be well-taken care of without worry.

In 2013, BDSRA engaged special education expert Lindsey Adams, and in 2015, Megan Orme joined to coordinate childcare. Lindsey and Megan work closely to ensure that affected loved ones in kids care have a good time during the conference, their dignity is kept intact, and most importantly they are loved and cared for.

To ensure a well-staffed ratio of nurses to kids, BDSRA hires nurses from Maxim Healthcare Services with the financial support of Our Promise to Nicholas. Lindsey and Megan organize an orientation with the nurses in each city, so staff understands the special needs of the children with Batten. When parents register their children, they provide information on favorite activities and interests that are used to tailor activities to the ability level of those registered. Activities include toys, games, coloring, art, movies, pet therapy, and music therapy.

New to the conference in 2017 was Chiltern Music Therapy, Directed by expert Rebecca Atkinson. Rebecca joined us from the U.K. with the support of Ethan's Reason and Harmony for Hope. She led singing, songwriting and a dance party in Affected Kids Care encouraging kids to let loose and have a good time. Rebecca also shared her work in Neurologic Music Therapy and its importance during a presentation open to all attendees at the conference. We continue to evolve Affected Kids Care based on feedback from participating families and make it the best possible experience for loved ones. When recently asked about the conference childcare parents shared their reflections:

"They do provide excellent childcare and can handle just about anything our kids could throw at them"  
– Heidi Bigelow

"It's a wonderful experience! As nice as it would be to have your whole family there, do what you are comfortable with. The care for affected children is top notch, and they are well prepared to handle anything and everything!" - Monica Bearman

We look forward to seeing you at BDSRA's 29th Annual Family Conference in Nashville, TN.



# Batten Without Boundaries

Finding lifelong friends is a wonderful thing – to find them while living with a Batten disease diagnosis has proven to be vital for many families. From the early days of a shared 800 telephone line and regional meetings to the social media and instant messaging of today, Batten families have formed friendships that span state and country lines.



Marlo Schinell from Virginia and Dixie Bergeron from Mississippi are two moms whose connection began on Facebook. Marlo reached out to Dixie when she heard of a new family with a Batten diagnosis, ready to share her knowledge and offer her support. "I can call on her for advice and comfort, and she is there for me... No judgment! That is extremely important as we know there is nothing normal at all about what we endure," Marlo says of their friendship.

Families also connect in-person at BDSRA's Family Conference. The Diaz family (Ohio), the Timko family (Pennsylvania), the Nelson family (Colorado) and the Gair Family (Canada) are one such example. The group talks weekly, sharing the daily aspects of their lives: work, school, marriages, and dreams. As Barbara Diaz explains it, "There's peace in knowing that we will all have good times and bad times, but we will never be alone." The siblings and dads have also found support and friendships. Batten siblings are a special group, being able to reach out to someone who fully understands their similar struggles and deep pain is priceless. These families don't just see each other at a conference, they extend their stays in the host city and vacation together as well!

Living in different countries didn't stop the James family (California) and the Brochu family (Canada) from quickly connecting and communicating on Facebook when they learned their children shared commonalities. "The friendships made in the Facebook group are invaluable. Never before had I had the chance to connect with another parent who completely – completely got it," shares Leah Brochu.

We are thankful when support programs like the family conference and closed Facebook page for primary caregivers provide a platform for families to connect. Our goal is that no family ever has to be in this alone and that they find a peer support system who will go through this journey with them.





## Thank You to Our Sustaining Donors of 10 Years

Your dedication to funding our mission allows us to focus on supporting families, advocating for our children, and advancing high quality scientific research. We are deeply grateful for your consistent financial contributions. These yearly gifts provide BDSRA the stability to provide excellent services to all Batten families and grow to meet the needs of our community in the future. Thank you to the loyal donors below who share our vision. We acknowledge your commitment to giving every year for the last decade.



### Batten Disease Support and Research Southeast Chapter

Margaret E Bingham

T. James and Maryalice Blake

Michael Caulfield and Lori Schaller

CFC United Way Of Central Ohio

Charles and Rose Chance

Susan Converse

Timothy and Kristin Coon

Robert and Corky Cooper

David and Corrina Dahl

Dennis and Carolyn Dahl

Dennis and Sharon Depoi

Gene and Edie Dockter

Elna Faret

Eric and Lisa Faret

Robert and Audrey Fink

Donna Fitzgerald

Melton and Marilyn Frederick

Gregg and Paula Froio

Mary A Gebhardt

Beverly Goll

Les and Debbie Ham

Heart Of America Chapter BDSRA

Daniel and Nancy Hynie

Bill and Diana Iltzsche

Alain and Janie Isnard

Keith and Alice Johnson

Lance W. Johnston

Daniel and Anne Kapp  
Wayne and Trina Kiefer  
Douglas and Susan Klee  
Charles and Wanda Leffler  
Martin and Vicki Lumm  
William and Mary Mapes  
Maugansville Ruritan Club/Kevin Lumm  
Memorial Fund  
Joel and Christine Maynard  
Metro NY/NJ Chapter BDSRA  
Richard and Clare Meyer  
William and Cynthia Milani  
Charles and Tini Montavon  
Neil and Leslie Nelson  
Sandra Phillips and Kristen Coon  
Nripendra and Suneeta Rana  
Brian and Nancy Reese

Larry and Jacquelyn Reichardt  
Randy and Darlene Royalty  
Charles and Anita Scanlon  
Cindy Schaller and Brenda Desena  
Richard and Linda Sheerman  
William and Patricia Shell  
Warren and Lois Shuros  
Martha C Skelly  
Madeline F Stone  
Steven and Amy Summers  
TCG Consulting, LLC  
Eric and Christine Thelen  
Bonnie Jo Thompson  
Stephen and Bonnie Thompson  
Anthony and Loretta Tomich  
United Way California Capital Region



# Thank You Donors!

Your contributions have impacted the Batten disease community in a big way. These are just a few of the key areas where your donations made a difference.

- Welcoming new families to the community and ensuring they have materials and connections to support their individual needs.
- Furthering research tools such as animal models and cell lines.
- Facilitating the first-ever FDA approved treatment for any form of Batten disease.
- Ensuring BDSRA can remain on the front lines speaking to key audiences to further awareness, education and action.
- Progress shortening the diagnostic journey.
- Continue the rich tradition of family support at the conference and year-round, whenever we are needed.
- BDSRA has received a Guidestar Gold rating as a charitable organization.







“BDSRA is a lifeline for families like ours. Literally.” – **Chris Hawkins**

“I like that BDSRA does not give up the quest to find a treatment or cure, and that they are there to support us. I like that they are truly growing the organization and helping to create awareness in many areas previously untouched.” – **Peggy Dicapua**

“I am a Batters Disease Child/Adult. I don’t know what life would be without you.” –  
Jonathon Brown – **Signed by Jonathon’s Mom, guardian and top supporter**

# Statement of Financial Position

## Batten Disease Support and Research Association / December 31, 2017

This financial information for 2017 has been summarized from the financial statements of BDSRA. A complete copy is available upon request from the Batten Disease Support and Research Association, 2780 Airport Drive, Suite 342, Columbus, OH 43219.

### Assets

Cash and Cash Equivalents	\$111,716
Investments	\$345,011
Equipment, less accumulated depreciation	\$83
Other Assets	\$5560
<b>Total Assets</b>	<b>\$462,370</b>

### Liabilities and Net Assets

Payroll and Associated Taxes	\$666
Accrued Expenses	\$5,638
Deferred Revenue	\$202,732
Total Liabilities	\$209,036
<b>Total Net Assets</b>	<b>\$253,334</b>
 <b>Total Liabilities and Net Assets</b>	 <b>\$462,370</b>

### Revenue and Support

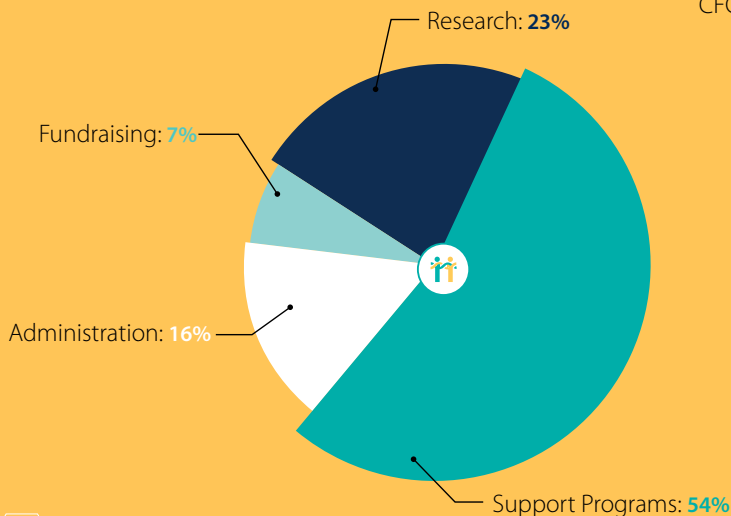
Donations	\$835,329
Dues	\$2,000
Interest/Dividend Income	\$78
Unrealized (Loss)/Gain on Investments	\$31,050
Net Assets Released from Restrictions	
<b>Total Revenue and Support</b>	<b>\$868,457</b>

### Expenses

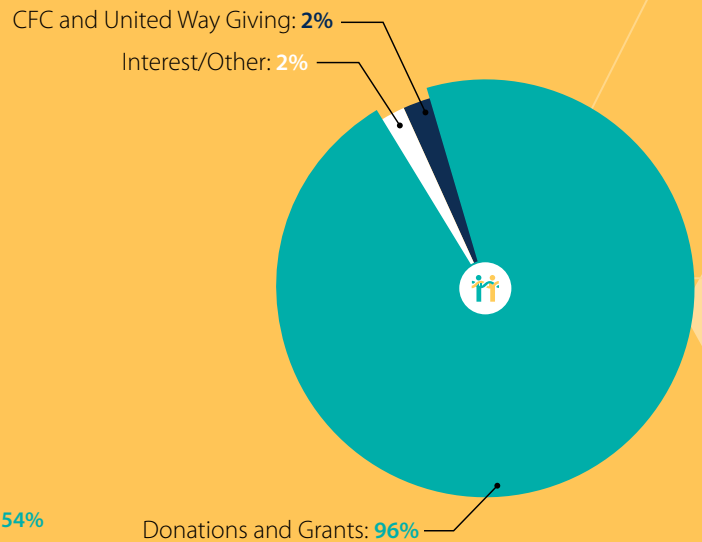
Research Grants	\$227,113
Other Program	\$498,842
Total Program	\$725,955
Fundraising	\$69,760
Management and General	\$160,253
<b>Total Expenses</b>	<b>\$955,968</b>

Change in Net Assets	(\$87,511)
Net Assets at Beginning of Year	\$340,845
Net Assets at End of Year	\$253,334

## Expenses by Category



## Revenue by Category



## Looking to the Future

BDSRA has a long list of ongoing initiatives – more than we can possibly accomplish in the upcoming year alone. We are a team, together with hundreds of families contributing in different ways. Looking ahead, we are deepening support services in newly emerging areas of need, launching a pilot of the Batten Family Help Fund and highlighting critical research needs. BDSRA carefully selects items that build upon the core values of the organization.

Our long-term vision is a world without Batten disease. Our mission is to support Batten families at whatever stage they are in their journey, fund and facilitate research, and advocate for treatments and a cure. As a family-centered nonprofit, the experience of the patient is at the heart of everything we do. The victories of 2017 fuel the hope we have for the year ahead. There are many ways you can support our community. Whether you donate, volunteer or share your story with others, we need you to make 2018 a year of significant progress.

[www.bdsra.org/donate](http://www.bdsra.org/donate)





**BATTEN DISEASE**<sup>SM</sup>  
Support and Research Association

2780 Airport Drive, Suite 342  
Columbus, OH 43219  
800-448-4570  
[www.bdsra.org](http://www.bdsra.org)