



**BATTEN  
DISEASE**

**SUPPORT & RESEARCH  
ASSOCIATION**

A LIGHT IN A WORLD OF DARKNESS

**Batten Disease Support  
& Research Association**

# **Hope on the Horizon Annual Report 2009**





**2008-2009**

**Help & Hope  
Change & Challenge**

## Message from our Director

### Board of Directors

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Dear Supporters:

This past year has been a year of change and challenge, yet there is hope on the horizon. On behalf of the Board of Directors, staff, volunteers, and those we serve, I am pleased to present the Annual Report for 2009.

We have given help and hope to hundreds of individuals and families who have been affected by Batten disease. Those affected are the reason we exist. The daily struggles of families in crisis motivate us to continue providing information, support and funding researchers who are our hope for effective treatments, and ultimately, a cure.

Until a cure is found, awareness and education remain a critical part of our mission. Thank you for your continued support which enables us to battle against this disease for the children today, and tomorrow.

Warmest Regards,

*Lance W. Johnston*

Lance W. Johnston  
Executive Director

# Medical & Scientific Advisory Board

## Medical Advisory Board

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Nationwide Children's Hospital

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Doernbecher Children's Hospital



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University of Texas Southwestern

Michael Brenner, PhD.  
University of Alabama

Jon Cooper, PhD.  
Kings College London

David Pearce, PhD. - Chair  
University of Rochester

Mark Sands, PhD.  
Washington University at St. Louis

David Sleat, PhD.  
Center for Advanced Biomedical Research



# Our History

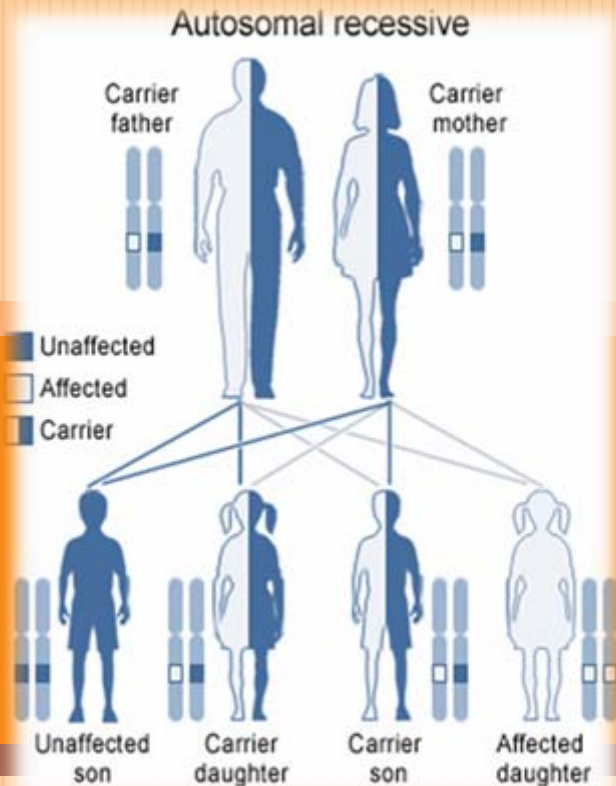
**Mission Statement:** to be an international support and research networking organization for families of children and young adults with an inherited neurological degenerative disorder known as Batten disease.

Our mission has changed little since the organization's inception in 1987, as we are still here for the hundreds of families whose children have or had this devastating genetic disease named Batten disease.

We are committed to raising the bar in fostering and funding research and offering unparalleled support for families as their stories are our inspiration. Additionally, to honor our donor's intentions, we continue to operate a very lean and efficient organization and are proud to state *that less than 9%* of our income goes to provide support for our annual operating and administration expenses.

As the financial report enclosed shows, we can only accomplish what we do because of our donors. Thank you for your partnership as we continue with our mission to ensure that there is hope and help for the families of tomorrow who will be fighting this disease.

# Fact Sheet



More than 120 families in 12 countries were supported with services this past year.

BDSRA is the largest private source of funds for international research on Batten disease.

- BDSRA was formed in 1987, by parents of three children with Batten disease in an effort to provide support for parents, provide education about the disease, and act as a national registry for NCL researchers around the world.
- BDSRA is the only family support organization in the United States, Canada, New Zealand, Australia, Spain, South America and South Africa which is specific to Batten disease.
- BDSRA maintains a comprehensive clinical database of children and their families living with Batten disease. More than 1,200 families are currently registered.
- Batten disease is a rare disease, occurring in an estimated 2 to 4 of every 100,000 births in the United States. There are currently 10 identified types of NCL / Batten disease.
- 60 new families were supported in 2008 and over 120 families worldwide received program information and services.
- BDSRA is the largest private source of funds for international research on Batten disease providing over \$300,000 last year in research awards and clinical trial funds.
- BDSRA directs over 89% of each dollar toward programs and services to directly support families.
- Nearly 49% of BDSRA revenue is derived from private donations.

**30 children died of Batten disease last year. There is no treatment, there is no cure...yet.**



# Family Programs

Our goal is to advocate for the children affected by this disease, and connect parents and caregivers with the information and support they need.

- **Our Parent Mentor Program** partners new families with an experienced family walking the same path as you are.
- **Our Sibling Support Program** gives non affected children an outlet and resource to discuss their own feelings, fears, and concerns about the disease. There is also a Sibling Carrier Testing program which assist siblings without insurance by paying for testing.
- **Our Grandparent Support Program** is a unique program run by grandparents themselves that provides information and networking as they face their own circumstances with Batten disease.
- **Our Education Support Program** uses special education teachers to provide information to educators and schools about Batten disease and make recommendations of how to best help the child.
- **Our Medical Referral and Assistance Program** includes a Medical Advisory Board, consultants, and our staff Registered Nurse to provide understanding of medical issues facing children with the disease.
- **The Equipment Exchange Program** allows families to honor children's lives by passing on equipment that helped their children and, at the same time, keep it "in the family".
- **The Tissue Donation Program** is operated in conjunction with the Human Brain and Spinal Fluid Resource Center and makes it possible for families to donate their child's brain and other tissues for research into Batten disease.
- **Our Bereavement Outreach Program** assist families to work through the grieving process when their child has died from Batten disease.



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# Support Services

What a family needs today, may be very different tomorrow.  
Our support is available for whatever is needed at any time.

- Our **800 Toll Free Hotline** is available to families, medical professionals and others who are seeking assistance, information and support.
- 20 Regional Chapters** in the U.S., Canada, Australia, South Africa, South American and Spain provide a more localized support network.
- A **Family Directory** provides names and information about families for families who wish to network.
- The BDSRA website [www.bdsra.org](http://www.bdsra.org) connects families around the world instantaneously
- The **Annual Family Conference** is the cornerstone of our programs. It brings together families from around the world along with doctors and scientists to meet, learn, and share experiences and knowledge.
- The Illuminator** is our quarterly newsletter and a place to share tips, trials, and tribulations as well as information on fundraisers around the country.
- Resource Publications** are available at no cost on a variety of medical topics, education, fundraising and general information about the disease.



The research being funded holds great potential and hope for families.

# Research Grants

## Is There a Cure?

Inevitably the first question asked when a family contacts BDSRA begins with *“Is there a cure?”* Today, we can say we are getting closer and that there is *“hope on the horizon”*.

**We provided more than \$136,000 in funding to researchers in 2008.**

### **2008-09 Research Initiative Grants**

**J. Alfred Rider Memorial Research Award:** Mark Sands, PhD, Washington University in St. Louis - *“Combination Therapy for Infantile Neuronal Ceroid Lipofuscinosis*

**Krystyna Wisniewshi Memorial Research Award:** Sandra Hofmann, MD., PhD., University of Texas Southwestern - *“Enzyme Replacement Therapy for Palmitoyl Protein Thioesterase Deficiency”*

Jonathon Cooper, PhD., Kings College London – *“Pathogenic impact of lymphocytes and macrophages in Infantile Neuronal Ceroid Lipofuscinosis.*

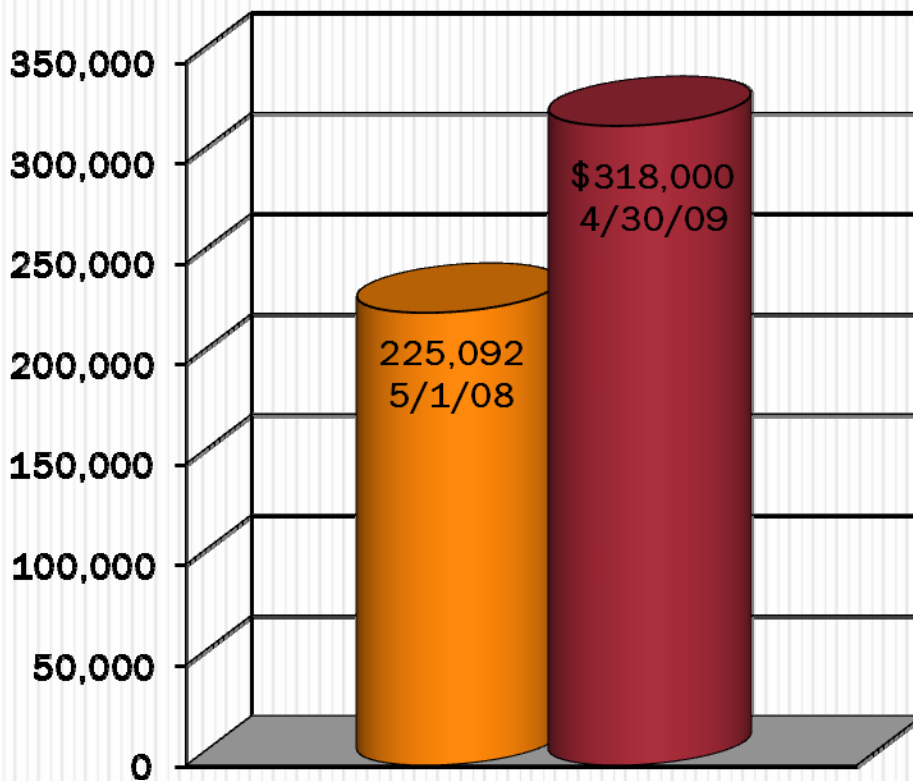
James T. Mapes, PhD., Rules Based Medicine, and David Pearce, PhD., University of Rochester, *“Identification of biomarker profiles associated with Batten disease using established mouse models of the NCL.”*

In July 2007, Batten Disease Support & Research Association began raising funds for a drug trial for Juvenile Batten disease. We established a goal of \$500,000 to be raised and provided to researchers by February 1, 2010. Estimated start date for the trial is January 2010. The drug will hopefully reduce some of the effects of the disorder.

# Juvenile Trial Fund

\$92,908 raised FY 2009

**\$318,000 Total**





**2009 Conference  
St. Louis, MO  
July 16-19**

**2010 Conference  
Chicago, IL  
July 29-31**

# Climbing Mountains Together

## 2008 Annual Family Conference

The 2008 “Climbing Mountains Together” Conference was held in Denver, CO. This 3 1/2 day conference provides socialization for our families and attendees, as well as important information about research and trial updates, education opportunities and a memorial service to remember all the children we lost this past year.

### **FUNDRAISING TOPS \$339,000**

#### **Individual Fundraising Awards**

##### **Research :**

- Taylor’s Tale-King Family \$90,061
- Pete and Joni Metcalf \$30,000
- Chris and Wendy Hawkins \$18,396

##### **Operations, programs and services:**

- Joe and Kathy Allio \$33,500
- Pete and Joni Metcalfe \$8,500
- Derek and Christie Allbee \$6,500

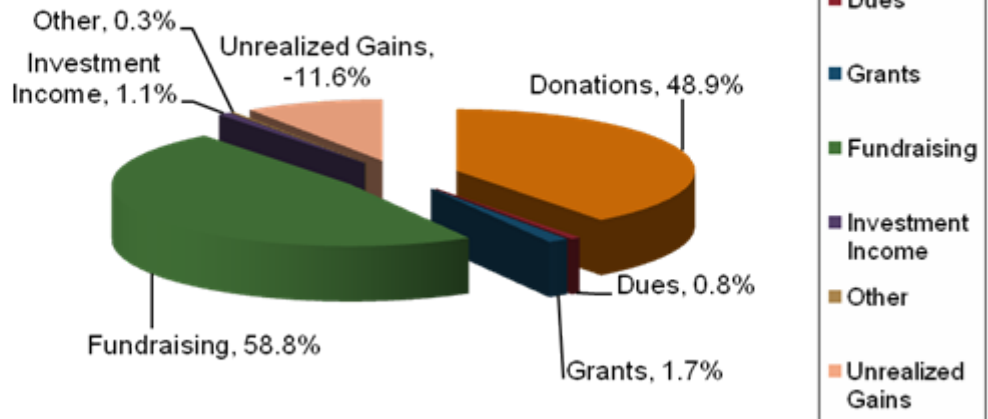
##### **Chapter combined :**

- Southeast Chapter \$116,500
- Northern California \$96,900
- Minnesota \$42,000

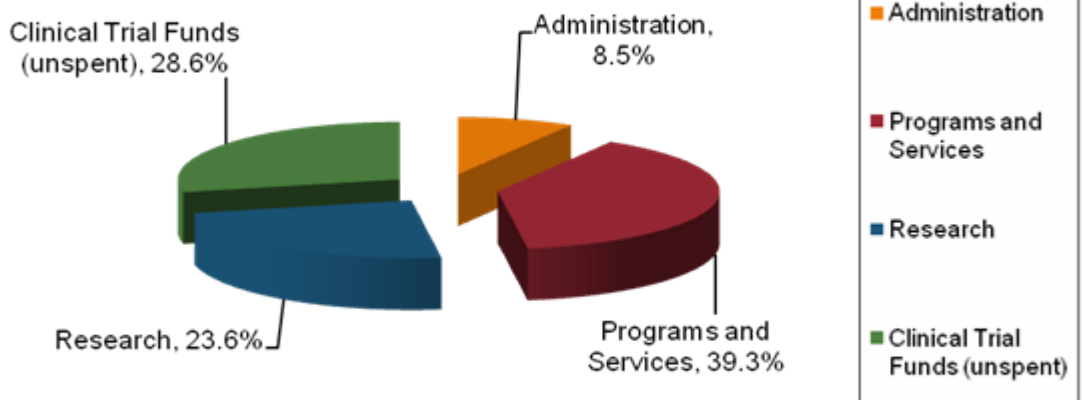


# Fiscal Year 2009 Financials

## BDSRA Revenue FYE April 30, 2009



## BDSRA Expenditures FYE April 30, 2009





# Appreciation and Support

Every donation received helps make our programs and services better and available to those who need them.

Whether you are a newly diagnosed family or a promising researcher, we are here for you.

Gifts to BDSRA are always welcome. In fact, they are vital for research and every program and service described in this Annual Report. Whether in the form of a cash donation, appreciated securities, a will, trust or charitable gift annuity, each contribution benefits our children.

## **Memorial and Honorary Gifts**

A gift given in memory of a loved one is a beautiful way of perpetuating that person's memory. Honorary gifts can also be given to recognize a special occasion in someone's life...birthday, anniversary, graduation, or wedding. Appropriate cards are sent to the family acknowledging your gift and thoughtfulness.

## **Bequests**

Remembering BDSRA in your will ensures that the work you cared so much about in your lifetime continues, even after your death.

## **Gifts of Appreciated Assets**

Gifts of assets that have increased in value since their purchase, such as stocks, bonds, mutual funds, and real estate, are particularly attractive under tax laws.

## **Life Insurance Gifts**

A gift of life insurance is easy to make and allows you to give more than may be possible otherwise.

## **Matching Gifts**

Does your employer or your spouse's employer have a charitable matching gifts program? If so, you may be able to double or triple the amount of your gift to us just by sending in a signed form with your donation.

For further information on giving, contact the Development Office at 866-287-7233. As always, your friendship and support are deeply appreciated.

As a small nonprofit organization, BDSRA relies on philanthropic support to advance its mission. Between May 1, 2008 and April 30, 2009 many donors made gifts large and small for which we are extremely grateful. Unfortunately, due to space limitations, only donors of \$250 or more are acknowledged here. We apologize if there are any omissions or misspellings. Please alert us to any changes by calling the national office at 866-287-7233 or by e-mail to [aryan@bdsra.org](mailto:aryan@bdsra.org).

# With Gratitude

The Batten Disease Support and Research Association wishes to express its deepest gratitude to the following members of the BDSRA family. Through the vision and leadership of these donors who have provided legacy gifts and donations of \$5,000 and up, BDSRA will be able to pursue the promise of research to contribute to a cure for Batten disease.

## Guardian Light Guild

*Donors who have left us in their estate plans*

*\*Denotes deceased*

Lars and Leilani Anderson  
Gladsia Bland\*  
Lance and Elaine\* Johnston  
Cecelia Martin\*  
Diane Plasko  
Barbara Tegen\*  
Dolores Toomey"

## Keepers of Hopes & Dreams

**\$25,000+**

Heart of America Chapter BDSRA  
Metro NY/NJ Chapter BDSRA  
Minnesota Chapter BDSRA  
Nor Cal Chapter BDSRA  
David and Martha Rickman  
SE Chapter BDSRA

## Advocates for Awareness

**\$10,000 to \$24,999**

Thomas Cornell  
Easter Foundation  
Emerson Charitable Trust  
Melissa Froio Foundation  
Karl Senner, Inc.  
Ontario Canada Chapter BDSRA  
John and Mary Turner

## Caring Hands

**\$5,000 to \$9,999**

Centa Corporation  
Jeremy and Dawn Dell'Aringa  
Bernadette Demoura  
Dennis and Sharon Depoi  
Heart of America Chapter BDSRA  
Ty and Kerrie Kurth  
Philip and Katherine Sproger  
Adam Weaver

## Circle of Friends

**\$1,000 to \$4,999**

Accelrys Software, Inc.  
Andrew Smith Memorial Fund  
Becky Lucas  
Barbara McDonough  
Beyond Batten Disease Foundation  
Michael Caulfield  
Rebecca Coffman  
Companion Veterinary Clinic  
CSX Corporation  
David and Corrina Dahl  
Dorothy Goforth  
Neil and Miranda Goranflo  
Ronald and Laurie Hudik

Keith and Alice Johnson  
Ross S. Johnson  
KFL Consulting Inc.  
Homer and Yvonne Midtlyng  
Robert and Marjorie Newell  
Northern Virginia Electric Corporation  
Ohio State Eagles Charity Fund  
Ray Family Foundation  
Riverview Baptist Church  
Stuart Tan  
Third Federal Savings and Loan

# With Gratitude

## Supporters

\$250-\$999

James and Anita Allardice  
Joseph and Kathleen Allio  
Alpha Chi  
Brian and Karen Anderson  
Lars and Leilani Anderson  
Nelda Mae Anderson  
Anheuser-Busch, Inc.  
B.P.O.E, Elks Lodge #2295  
Wendy Bills  
Tim and Maryalice Blake  
Robert Bobb  
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Brianna's Beads  
Dean and Alice Bucalos  
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Harold and Carol Busse  
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Diageo North American Foundation  
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Casimer and Patricia Durava  
Graciela Eberhard  
Leonard and Margot Farrelly  
Donna Fitzgerald  
Florida Chapter BDSRA  
Fosnaught-Holdship Funeral Home  
Paul and Linda Frank  
Kevin and Angelique Gardeck

Chris and Kristin Gaughan  
General and Implant Dentistry  
Tammy Gulgren  
Ryan and Jennifer Hare  
Paul and Linda Frank  
Kevin and Angelique Gardeck  
General and Implant Dentistry  
Tammy Gulgren  
Ryan and Jennifer Hare  
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James and Linda Smith  
Kohl's  
Mack and Caroline Lancaster  
Landmark Construction  
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Chad and Shannon Leis  
William and Elizabeth Mack  
Joel and Christine Maynard  
Bruce and Annette Meier  
Mendel and Co. Construction  
Metropolitan Arts Partnership  
Richard and Clare Meyer  
James and Becky Miles-Polka  
James and Joan Miller  
Miscere, Inc.  
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O&S Development, Inc.  
Office of Vicar for Priests  
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Michael and Victoria Pinder  
Pride International, Inc.

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