



Dear Families and Friends

BDSRA Australia is thrilled that Brineura, the ground-breaking treatment for children with late infantile batten disease (CLN2), has been included in the Life Saving Drugs Program. We commend the Australian Government, the Chief Medical Officer and the Expert Panel of the Life Saving Drugs Program for recognising the value and importance of this treatment. Vanessa Anderson, our public officer and long standing champion of the Australian chapter of the BDSRA, has been fundraising for Batten disease research since 1997. She says:

"This is truly wonderful - I never thought I'd actually hear the words 'a treatment for Batten disease'... There were some (all very important) hit and miss trials and I realised a treatment wouldn't be in my Jordan's short time on this earth. ... I wasn't even sure if I was going to see this milestone in my lifetime. I like to think that my Jordan (CLN2), together with many others who passed from Batten disease are pioneers for such new treatments...A cure for all forms of Batten disease IS possible!"

A huge thank you to all of our families who shared their stories and to our pioneers who gave up everything to ensure that no other CLN2 families have to suffer this disease without treatment.



Vanessa Anderson, BDSRA Australia public officer and Batten disease warrior

NEW Medical and Scientific Advisory Board for BDSRA Australia

We are delighted to announce the formation of BDSRA Australia's inaugural Medical and Scientific Advisory Board, comprised of leading medical and allied health experts with experience in diagnosis, treatment and management of Batten Disease. The Advisory Board is primarily a resource for other healthcare practitioners seeking advice on Batten disease management. The members are:

Dr Alex Johnson (Paediatric Neurologist, Sydney Children's Hospital) (Chair)

Professor Ingrid Scheffer AO (Director Paediatrics, Austin Health Melbourne)

Professor Monique Ryan (Director Neurology, Royal Children's Hospital Melbourne)

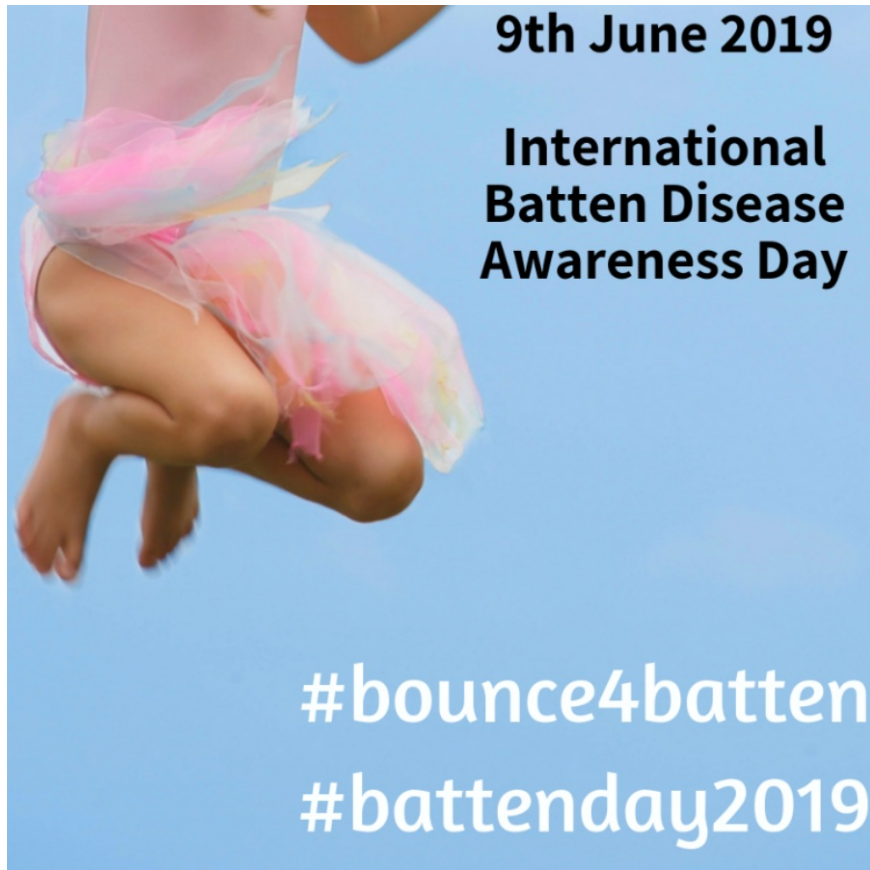
Professor John Grigg (Ophthalmologist & Director of the Save Sight Institute, Sydney University)

Associate Professor Carolyn Ellaway (Head of Metabolic Genetics, Children's Hospital Westmead, Sydney)

Dr Nick Smith (Head of Neurology, Women and Children's Hospital Adelaide)

Cathy Horder (Senior Paediatric Occupational Therapist, Melbourne)

International Batten Disease Awareness Day - 9 June 2019



International Batten Disease Awareness Day is Sunday, 9 June 2019. It is a great day to raise awareness about Batten disease. Have you considered sharing your story of Batten disease with your local newspaper? Or asking your local school to #bounce4batten and post a picture on social media? Please do something special to honour the children and families affected by Batten disease. For more information see www.bounce4batten.com, or share our facebook link [here](#).

BDSRA Australia Parents and Carers Lunch - Sydney, 14 July 2019

Attention all parents/carers of children with Batten disease - would you be interested in attending a 'catch up' lunch in Sydney on 14 July 2019? A travel allowance would be available for those living interstate and outside of Sydney. Further information was sent to families directly by email last week. If you did not receive an email, please contact info@bdsraustralia.org.

Tom the 'Bin Truck' driver

TLC for Kids made Tom's dream come true by arranging a 'bin truck' experience. TLC for Kids is dedicated to giving practical and emotional support to sick kids and their families across Australia. Read the full story [here](#) and see <https://www.tlcforkids.org.au/>



Batten disease research - Dr Tony Cook, University of Tasmania

Dr Tony Cook at the University of Tasmania leads one of the research projects funded by BDSRA Australia. He has been using stem cell technology to study how the CLN3 and CLN2 genes affect nerve cell health in Batten disease. You can read his interim report [here](#).

Useful Links and Resources

- **Review article:** “*Therapeutic landscape for Batten disease: current treatments and future prospects*” Nature Reviews Neurology, 15, pp 161 –178 (2019), provides an overview of the promising therapeutic avenues for Batten disease, including current FDA-approved clinical trials and prospective future treatments. See key points here: <https://www.nature.com/articles/s41582-019-0138-8>
- **CLN3** - a new book, titled ‘*Juvenile Neuronal Ceroid Lipofuscinosis, Childhood Dementia and Education: Intervention, education and learning strategies in a lifetime perspective*’ (January 2019, Norway), addresses learning strategies for students with juvenile Batten disease based on worldwide research. Contact Bengt Elmerskog belmerskog@gmail.com to obtain a copy.
- **CLN2** - two recent medical publications describe, respectively, CLN2 disease characteristics and progression (the natural history); and management strategies for CLN2: (1) Nickel et al., “*Disease characteristics and progression in patients with late-infantile neuronal ceroid lipofuscinosis type 2 (CLN2) disease: an observational cohort study*”, Lancet Child Adolesc Health 2018; and (2) Williams et al., “*Management Strategies for CLN2 Disease*”, Pediatric Neurology 69 (2017) 102-112. These publications can be useful in NDIS meetings to explain the disease and the expected needs of CLN2 children as the disease progresses.

News

A step closer to gene therapy for CLN1

Abeona Therapeutics has announced that ABO-202 gene therapy for the treatment of children with CLN1 disease has received FDA clearance for a Phase 1/2 clinical trial. The global trial of ABO-202 is expected to begin in the US and Germany. Please see [here](#) for more information.

Library catalogue

BDSRA Australian now has a library catalogue. You can access it [here](#). Please contact Julie on info@bdsraustralia.org to obtain access to publications.

BDSRA United States - family conference, 18-21 July, Colorado

The US chapter of the BDSRA will be holding their family conference in Denver, Colorado. Join the facebook group below for further information and updates if you are interested in attending: www.facebook.com/BDSRAFamilyConference

Fundraising

ASX Refinitiv Charity Foundation

BDSRA Australia has been chosen as an ASX Refinitiv Charity Foundation charity partner for 2019. Please consider purchasing a ticket or, even better, a table at the Australia's biggest Charity Horse Racing Day, at Rosehill Gardens Racecourse, Sydney on 27th July 2019, hosted by the Foundation. The more tickets we sell– the more funds BDSRA Australia will receive. Please find information here: [Charity Horse Racing Day](#)

Our Everyday Heroes - “nobody can do everything, but everyone can do something”

Have you considered raising money for BDSRA Australia? It is easy with “Everyday Hero”, which can turn any event, large or small, into a fundraiser, with direct donations to BDSRA Australia. All you need to do is create an Everyday Hero account; choose “**Batten Disease Association**”; and then your supporters can donate directly to BDSRA Australia via a link that will be sent to you. To learn more or create your own Fundraising Page with our charity as your choice, follow this link:

<https://give.everydayhero.com/au/get-started>

You can support our current Everyday Heros here:

[Julie Ojeda - Tough Mudder](#)

[Meg Garrido - City to Surf](#)



Meg Garrido, a dedicated BDSRA Australia volunteer and an 'Everyday Hero'

Donations

Thank you to everybody who donated to BDSRA Australia recently, with a special acknowledgment to our largest recent donors: Mr Dean Apostolou, Mr Vallejo Gantner, Mr Dashiell Gantner and #Bottles4Batten. #Bottles4Batten is the wonderful effort of Mr Andreas Mickler and the students of Trinity Grammar School in Sydney and West Ryde Anglican Church in collecting and recycling bottles to raise money for BDSRA Australia.

We also wanted to express our extreme gratitude for the long term generosity of Mr Apostolou, who has been providing support to BDSRA Australia for many years after hearing about the plight of families affected by Batten disease.

Without your support, we would not exist. You can support BDSRA Australia by giving at

<https://www.givenow.com.au/battendiseasesupport>