

NEWSLETTER

Summer 2023 Edition



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Amber Rundle,
BDSRA Australia
President

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President's Report



As in-coming President of BDSRA Australia, I'd first like to say a huge thank you to our outgoing members, former President **Kate Beattie** and former Treasurer **Mandy Meredith** for their outstanding work and commitment to BDSRA Australia over the past few years, and for their continued support during this 'handover' phase. I speak for all of the Board and management committee when I say we will miss you both dearly!

We welcome to the Board three new members - **Amanda Gilpin** (WA) who has moved from a General Board position to Treasurer, **Alicia Brown** (SA) mum to Kiaana (CLN3) and a passionate and knowledgeable advocate, and **Heidi Dowse** (QLD) who formerly led the Dementia Support Australia Childhood Dementia Support program, which was established in partnership with BDSRA Australia in 2019. Joining our management committee, we welcome **Bettina Lavington** (SA), mother of her precious Batten Angel Holley. Bettina carried out some remarkable fundraising efforts on behalf of her daughter and we look forward to getting her involved in all our fundraising initiatives.

We are also thrilled to announce the appointment of **Associate Professor Tony Cook** to the BDSRA Australia Medical and Scientific Advisory Board, which you can read more about below.

Since our last newsletter a lot has been happening! Most notably, we finally held our long-awaited **Family Retreat** in Sydney in November 2022, which saw over 60 attendees, including 18 Batten families from across the country come together to connect and to hear from some of our leading Batten disease clinicians, researchers and support providers.

The two-day program was jam-packed with educational sessions and social activities, including some memorable and much-deserved family fun time for our affected children, siblings and parents. We enjoyed inclusive ice-skating, a magic show, animal farm and wildlife animal encounter, movie night, arts and crafts, and arcade games. On the Sunday morning we took time to reflect in a beautiful and moving remembering service for our Batten angels.

For those who couldn't make the Retreat this time, you can still access the recorded educational presentations and check out our highlights reel, links in Events section. A special thanks again to all our wonderful sponsors and supporters (see below), and guest speakers who generously donated their time and expertise - **Dr Alex Johnson, A/Prof. Carolyn Ellaway, A/Prof. Tony Cook, Gemma Niwha, Hayley Curran, Jess King**, and all our family panelists.

In December we announced the winners of our **BDSRA Australia Research Grant 2022** round. I am proud to say, since the Research Grant Program began in 2020, we have now awarded more than \$400,000

toward world-class Batten disease research right here in our ANZ region. A remarkable achievement that has been made possible through the generosity and support of our community.

With 2023 now well and truly underway, we are in the midst of planning a number of great opportunities for families to connect and to access the latest updates and information through our successful '**Virtual Coffee Catchups**' and '**Meet the Expert**' webinar series, so please stay tuned for further info and remember to check our [Facebook](#), and other social media pages, and our website [News page](#).

Lastly, if you haven't already, I urge you to buy a raffle ticket in our **ASX Refinitiv raffle** which will be [closing this week at midnight on Friday, 3rd March](#). Proceeds from every ticket sale go directly to BDSRA Australia. To purchase your tickets and be in the running for some fabulous prizes, simply [click here](#) (or see further details below). Good luck and thank you for your support!

Amber

To read a brief bio on our new **President Amber Rundle**, head to our [About Us](#) page!

Research Update

Compiled by Dr Ineka Whiteman, Head of Research and Medical Affairs

Congratulations to our 2022 Research Grant Winners!

In December we announced three winners of the 2022 grant round, with a total of over \$120,000 awarded. Congratulations to chief investigators **Lottie Morison** from the Murdoch Children's Research Institute (\$13,950) for work in characterising speech and language deficits in Batten disease; **Dr Nadia Mitchell** from Lincoln University NZ (\$54,545) for her continued work in CLN5 and CLN6 gene therapy; and **A/Prof Tony Cook** from University of Tasmania (\$53,612) for his continued work in CLN3 stem cell models.

This year we are also grateful to have partnered with the **Beyond Batten Disease Foundation** to co-fund A/Prof. Cook's work. Read more about these new projects [here](#).



Lottie Morison
Murdoch Children's
Research Institute, VIC

Dr Nadia Mitchell
Lincoln University, NZ

A/Prof. Tony Cook
University of Tasmania



Dr Ineka (right) with BDSRA Foundation and BDFA colleagues at NCL Chicago

Conference, Chicago

In November, I was delighted to attend the **Translational Research Conference for the Management of NCLs** in Chicago. Held every two years, this meeting brings together leading NCL researchers, clinicians, industry and advocacy partners from around the world, alongside experts from outside the NCL field who bring fresh perspectives and novel insights from other technical disciplines and therapeutic areas. For a summary of key updates and insights from the meeting, download a copy of the **Research Report for Families** [here](#).



CLN3 disease – ‘Batten-1’ clinical research update

The **Beyond Batten Disease Foundation** (BBDF) together with industry partner **Theranexus** announced the initial results of their Phase I/II clinical trial for juvenile Batten disease (CLN3).

After a 9-week course of **Batten-1** (active ingredient miglustat) with the maximum dose of 600 mg/day, the first results of the Phase I/II trial enrolling six patients with CLN3 disease aged 17 and over showed good patient safety and tolerability, and a pharmacokinetic profile in line with expectations. A Phase III paediatric study is expected to commence later this year. Read more [here](#).



CLN2 disease – Gene therapy clinical research update

REGENXBIO has announced two important, positive milestones in their CNS and ocular gene therapy programs for the treatment of CLN2 disease:

1. Patient dosed under a single-patient investigator-initiated study of **RGX-181 (CNS-directed gene therapy)**.
 2. Approval of CTA for **RGX-381 (ocular gene therapy)** from the UK Health Authority and plans to initiate a Phase I/II clinical trial in the first half of 2023.
- Read more [here](#).

CLN7 disease - Statement from Global Batten Disease Organisations



Udruženje građana za borbu protiv retkih bolesti kod dece

Earlier this month, **Neurogene Inc** announced they were discontinuing their CLN7 disease research program. We believe it's important to work together with our global patient advocacy partners to share these major updates in a unified, timely, and factual manner. We released [this collaborative statement](#) to our Batten communities worldwide.



A/Prof Tony Cook

Advisory Board Appointment

We are delighted to announce a new appointment to BDSRA Australia's Medical & Scientific Advisory Board. **Associate Professor Tony Cook** is Head of the Stem Cell Models Group at the Wicking Dementia Centre, University of Tasmania. Read more about Tony [here](#).

Events

Upcoming

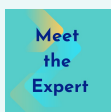


Virtual Coffee catchup - General Catchup

Join us for a relaxed chat, an opportunity to meet other parents from around Australia and NZ, to discuss topics that attendees raise.

Wednesday, 15th March - 1:00 PM - 2:30 PM (AEDT)

Registration link will be emailed and posted on Facebook very soon!



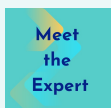
Meet The Expert - Managing Caregiver Stress & Burnout: The Importance of Self-Care

With special guest **Dr Joe Vigil**, Psychologist and Batten parent.

Live from New Orleans, USA

Saturday, 25th March - 10:00 AM - 11:00 AM (AEDT)

Registration link will be emailed and posted on Facebook very soon!



Meet The Expert - Managing challenging behaviours in Batten disease and childhood dementia

Join the Dementia Support Australia **Childhood Dementia Support team** as we discuss the behavioural and cognitive aspects of Batten disease and practical strategies to support children, their families and caregivers.

Wednesday, 3rd May - 8:00 PM - 9:00 PM (AEST)

Registration link will be emailed and posted on Facebook



Bounce4Batten & International Batten Disease Awareness Day

Please note the date in your diary - 9th June! We'll be

reaching out in April for families who might like to share their story for our social media campaign.

9th June 2023

Past



Virtual Coffee catchup - Feeding Tubes

In light of FEEDING TUBE AWARENESS WEEK earlier this month (5th Feb - 11 Feb) families joined us on Wednesday 1st March to discuss, ask questions and share experiences on this topic.



Virtual Coffee catchup

On Friday, 25th November we had a general zoom catchup. We had a lively chat covering varying topics that filled into overtime - there was so much to talk about, everything from getting to know new people, and sharing lived experiences to NDIS and latest research! Thanks to those who joined us.



Family Retreat

Our 2022 family retreat was a HUGE success. We had an amazing time coming 'together again' on the 2nd weekend of November.

Families left the retreat with renewed connections, full buckets and continued hope for our Batten warriors.

To see the weekend's highlights reel, click [here](#).

To watch Tony Cook's recording, click [here](#).

To watch Clinical Trial update recording, click [here](#).



With a special thanks to our sponsors and supporters for the 2022 Family Retreat

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Family Support



Rare Disease Day

On the 28th of February, we joined forces with the Rare Disease Day movement to raise awareness and promote change for over 300 million people worldwide living with a rare disease.

Did you know?

- ➔ A disease is classified as rare when it affects fewer than 1 in 2000 people.
- ➔ 70% of rare diseases start in childhood.
- ➔ Around 5% of the world's population have a rare disease.
- ➔ Around 95% of rare diseases have no effective treatment.

Right now, in Australia there are more than 50 children and young people living with Batten disease. Pictured here are some of our Batten warriors championing our cause for this year's Rare Disease Day.

RARE is MANY, RARE is STRONG, RARE is PROUD.

#rarediseaseday2023 #battendiseaseisrare #LightUpForRare
#ShareYourColours

To share your family's experience in the newsletter or for further information on how we can support you, please contact Liz at support@bdsraustralia.org

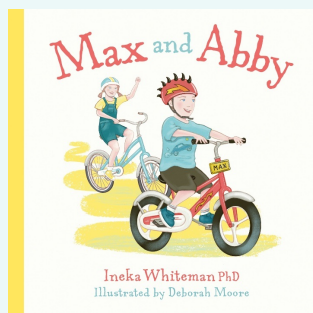
Our Batten Angels

Remembering Service

We remembered every one of our 102 Batten Angels from Australia and New Zealand in a moving service at our Family Retreat in November.



Resources



Children's book about Batten disease

Told through the eyes of big sister Abby, **Max and Abby** shines a gentle light on the challenging topic of childhood dementia and regressive disability, revealing the highs and lows of life as a sibling of a child with additional needs.

Have you ordered your copy?
Read more [here](#)



challenges in Batten disease

Dementia Support Australia offers free, nationwide specialised Childhood Dementia Support with trained consultants who understand childhood dementias like Batten disease, its related behavioural and cognitive challenges, and the impacts these can have on the child, their family and carers.

No referral needed. Simply call our 24-hour helpline on 1800 699 799, fill out the contact form or chat with us online. To learn more, visit us [here](#).

Not on social media?

Keep up-to-date on BDSRA Australia's latest news and events on our website!

We frequently add items to our website [News page](#), so feel free to head over anytime to check out the latest in research and support news!

Donations

Thank you so much for your support!

A big hello from the Treasurer's desk! For those that may not know me, my name is **Amanda Gilpin** and I recently came on Board as Treasurer for BDSRA Australia. I have very big shoes to fill, with **Mandy Meredith** taking her well-deserved retirement after more than 5 years in the role. I would love to take this opportunity to sincerely thank Mandy for the countless hours of hard work and dedication to BDSRA Australia over the years. She will be greatly missed!

We'd also like to recognise the incredible support and ongoing generosity we've seen from our Batten community in recent months. Since December 1, we've had more than \$13,000 in donations! We can't achieve our vision and goals without this ongoing generosity - so our sincerest THANK YOU!

[Donate Now](#)



In lieu of flowers or gifts

We thank those who recently donated to BDSRA Australia in lieu of flowers at the funeral of **Gordon Sanson** (grandfather of **Batten Angel Matilda Sanson**).

Over the years families have

Example photo of wishing well for charity donation in lieu of wedding gifts

encouraged donations for BDSRA Australia through family events such as funerals and birthdays. Here is another example of encouraging donations in lieu of wedding gifts.

Fundraising

ASX Refinitiv

In February the **ASX Refinitiv Foundation** held their annual Golf Tournament and Dinner. BDSRA Australia are privileged to be one of the ten charities supported by this foundation. A big thank you to the volunteers in attendance and families that supplied auction prizes.

Over the year we will be asking for more volunteers to help at these events and we are still in need of more auction prizes for the next event. If you can help please get in touch with Nikki at info@bdsraaustralia.org

Raffle tickets for the ASX Refinitiv Charity Foundation Raffle 2023 are now available!

HURRY! Raffle closes tomorrow, **Friday 3rd March 2023** and is drawn on 9th March 2023.

[Purchase Raffle Tickets](#)



Wilson Hickey and Bobbie Riddle

Fundraising Inspiration!

During the 2022 Family Retreat, BDSRA Australia Committee member **Bobbie Riddle** spoke to our delegates about the importance of fundraising and creative ways to fundraise.

Guest speaker **Wilson Hickey** was recognised for his SENSATIONAL efforts raising over \$7000 by cycling and swimming his way around Lord Howe Island to raise funds and awareness for Batten disease! **Thank you for your inspirational achievement, Wilson!**

If you are interested in hosting a fundraising event or activity to help support BDSRA Australia, or would like some ideas to help kick-start, please get in touch with us at info@bdsraaustralia.org

If you have a family member or friend fundraising for BDSRA Australia and are happy to share with us for our next newsletter or on our social

media, please let us know!

Personal Details Update: For all our families, so we may keep you informed and up to date, if your circumstances or contact details have changed, please inform Nikki Hopkins at info@bdsraaustralia.org



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