

NEWSLETTER

Autumn 2023 Edition



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Amber Rundle, BDSRA
Australia President

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President's Report



Amber with Anita Pandit (Right) from London Stock Exchange Group, receiving ASX Refinitiv Charity Foundation Cheque.

On Friday, 19th May I attended the ASX Refinitiv Charity Foundation's "2023 Charity Cheque Presentation and Sponsors Thank You Lunch". The luncheon was a fabulous opportunity to meet and show our gratitude and thanks to the generous donors that allow ASX Refinitiv Charity Foundation to continue the amazing fundraising they do on our behalf. It was also a great opportunity to meet some of the other very deserving charities supported by the initiative and hear their stories.

As you may already be aware from our recent Fundraising Update email correspondence we have once again been selected as one of the lucky charity recipients for the ASX Refinitiv Charity Foundations 2023-24 program. This makes such a significant impact towards us being able to continue our mission. Thank you to all our supporters and everyone who helped us achieve our raffle tickets sales targets to be able to retain this position.

Next **Friday, 9th June** we reach our most significant day on the calendar year with **International Batten Disease Awareness Day** where we all Bounce4Batten. We have been very busy behind the scenes preparing materials and information to have at the ready for next Friday. You may have noticed our social pages have been filled with inspiring and heartfelt stories from our courageous Batten community families and hopefully you've all been receiving our recent email correspondence about Fundraising and Bounce4Batten and how you can so easily get involved. Further details can be found in the fundraising section of this newsletter.

We have already heard of some terrific fundraising events that will be taking place including in South Australia, the 1st Goolwa Sea Scouts will be fundraising at Inflatable World on behalf of the McWaters family. I don't think there could be a more appropriate place to Bounce4Batten, what a great idea!!! We can't wait to hear how it goes.

Since our last newsletter we have sadly lost two of our Batten Warriors, Max Hallum and Georgia Gregg. As we reflect on their precious, but all too short lives, we approach this coming Friday with a true sense of purpose and a sober reminder of why we raising awareness and why getting the message out there is so crucial!! We thank you in advance for your support and efforts and can't wait to see everyone's social pages in flight and bouncing next **Friday 9th June**.

Lastly, I encourage any of our Batten Families who haven't yet done so to

fill your details in on our family register. Details on what to do are contained in this newsletter and it will only take you 2 minutes.

Amber

Research Update

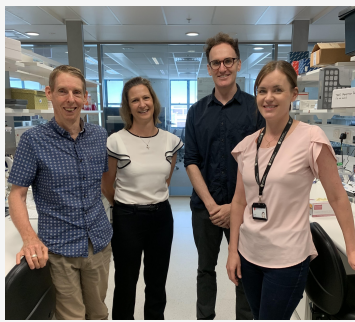
Compiled by Dr Ineka Whiteman, Head of Research and Medical Affairs

BDSRA Australia takes part in premier international meeting

It was a privilege to attend the Gordon Research Conference (GRC) for Lysosomal Diseases, from May 15-19 in Barcelona, Spain. GRCs are premier, international scientific conferences focused on advancing the frontiers of science through the presentation of the very latest, cutting-edge research. This year's theme was 'Connecting Lysosomal Function to Pathophysiology for Innovative Therapies'. The program included a diverse range of speakers and discussion leaders from institutions and organizations worldwide, including numerous talks and academic posters from international NCL experts presenting some of the most recent (and largely unpublished) scientific and clinical data from the NCL field.

It was wonderful to spend time with fellow attendees Dr Angela Schulz (right), Head of the NCL Specialty Clinic at University Medical Centre Hamburg-Eppendorf, Germany, and Dr Frank Stehr (left), CEO of Germany-based NCL research and advocacy organisation NCL Stiftung, discussing the latest in clinical trials, scientific research and patient support resources. While at GRC, I was delighted to gift a number of copies of BDSRA Australia's children's book [Max and Abby](#) and have been humbled by the positive response and follow up requests for its translation into several languages (stay tuned for further details!).





Research visit to QIMR

I recently enjoyed a visit to the QIMR Berghofer Medical Research Institute in Brisbane to meet with BDSRA Australia Research Grant recipient Associate Prof. Tony White (pictured left), and team members Dr Zachary Gerring (middle right) and Dr Lotta Oikari (far right). The team are currently undertaking a 'drug repositioning' project to identify potential treatments for Batten disease, utilising computational and bioinformatic approaches, combined with in vitro cell model research. The outcomes of this project may potentially provide a rapid pathway for identifying drugs that could then be tested in advanced clinical trials in patients with Batten disease. Read more [here](#).



Research Grant Program 2023-24 Round - Now open!!

BDSRA Australia is pleased to announce the 2023-2024 round of the Batten Disease Research Grant Program for Australia and New Zealand is now open.

Each year, BDSRA Australia invests in promising local research through its Batten Disease Research Grant Program. The objective of the program is to advance research across Australia and New Zealand.

BDSRA Australia typically awards up to AU\$100,000 in total each year, across one or more projects.

We are currently inviting Expressions of Interest for the 2023-2024 round.

Submissions close Wednesday 28th June 2023 at 5pm (AEST)

Read more [here](#).

Clinical Research Update



IMPORTANT NOTE TO FAMILIES: If you are

CLN3 disease – 'Batten-1' clinical trial

The Beyond Batten Disease Foundation together with industry partner Theranexus recently met with the FDA following the end of Phase 1/2 (a safety study) for the Batten-1 clinical trial. The investigational drug 'Batten-1' is a novel and proprietary

interested in participating in the Batten-1 clinical study, and your child has been diagnosed with CLN3 disease and is currently aged 4-16 years (or turning 4 in 2023-24), we recommend you fill in [this online form](#) to register your interest, as soon as possible.

If you have any questions, please feel free to reach out to Dr Ineka Whiteman, Head of Research and Medical Affairs any time at research@bdsraustralia.org

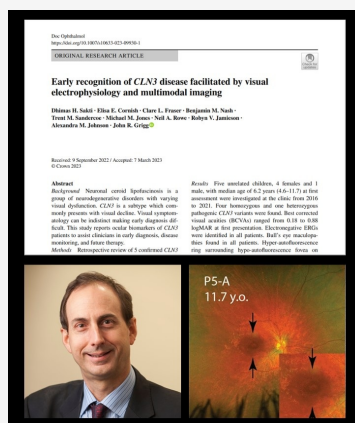
drug containing the active ingredient miglustat.

In the FDA meeting, plans to commence the Phase 3 of the clinical trial in paediatric patients (age 4-16 yrs) were discussed. The FDA have now given clear guidance to BBDF and Theranexus to finalize the Phase 3 protocol and to begin determining research sites. A contract research organisation (CRO) will now commence feasibility studies at sites around the world to determine which sites will be best suited for the trial. BBDF/Theranexus anticipate this study to be open for enrolment by the end of the year.

Read more [here](#).

Research Publications - Selected

A selection of recent research publications in Batten disease are provided below. For summaries and links to each article [click here](#).



CLN3 disease: ophthalmological findings are crucial to early diagnosis

A study led by [BDSRA Australia Medical Advisory Board](#) member, Prof. John Grigg (pictured) has revealed several ocular biomarkers (biological markers) specific to CLN3 disease that may assist clinicians in making timely, early diagnosis, and assist in monitoring ongoing disease progression. Read more [here](#).



Gene therapy rescues spontaneous seizures in CLN2 mouse - now in human trials

Seizures are one of the primary symptoms of CLN2 disease, but exactly why these occur has remained a mystery. This study shows for the first time that mice carrying one of the most common CLN2 mutations seen in human patients also have spontaneous seizures, often so severe they are fatal. Treatment with gene therapy prevented seizures, improved walking ability and prolonged lifespan in CLN2 mice. Read more [here](#).



Understanding protracted CLN3 disease; a milder, rarer form

A new study, co-authored by [BDSRA Australia Medical Advisory Board](#) member Prof. Ingrid Scheffer, has

characterised seizures and other clinical features in a cohort of patients with the rarer protracted form of CLN3 disease, in order to improve recognition and early diagnosis of the condition. Read more [here](#).



Changes in brain metabolites may be biomarkers for CLN3 disease

This recent study has shown that changes in the levels of certain metabolites in brains of people with CLN3 disease are significantly associated with multiple measures of disease severity. Measured by non-invasive magnetic resonance spectroscopy (MRS), these biological markers may be useful as surrogate measures of treatment responses in future clinical studies. Read more [here](#).



Lack of CLN3 harms the process of survival for cells under stress

This cell-based study suggests that absence of CLN3 protein – the underlying cause of CLN3 disease – leads to poor regulation of cell metabolism and impaired function of protective ‘stress granules’ (SGs), which are important for promoting cell survival under stressful conditions. Read more [here](#).



Modified stem cell therapy extends survival in CLN1 mice

In this study, treatment with stem cells that had been modified to produce PPT1 — the missing enzyme in CLN1 disease — prevented symptoms from developing and substantially extended survival in a mouse model of the disease. Read more [here](#).

Events

Upcoming



Virtual Coffee catchup - General open-topic Discussion

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

Monday, 5th June - 1:00 PM - 2:30 PM (AEST) [Register here](#).



International Batten Disease Awareness Day & Bounce4Batten

Let's raise awareness and take the opportunity to fundraise. Use social media to share stories, facts and bouncing photos. To find out more on how to participate, see our easy steps guide at the bottom of this newsletter. Please note the date in your diary.

Friday, 9th June 2023

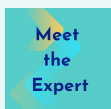


Virtual Coffee catchup - Accessible Vehicles

Join us for a discussion about everything vehicles, from fundraising to modifications through NDIS! Please bring along your experiences and questions to discuss with other families.

Monday, 19th June - 1:00 PM - 2:30 PM (AEST)

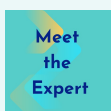
Registration link will be emailed and posted on Facebook very soon!



Meet The Expert - Understanding speech and language deficits in Batten disease

Join Speech Pathologist **Lottie Morison** and **Prof. Angela Morgan** from the Murdoch Children's Research Institute (MCRI) Melbourne, as we discuss speech and language challenges in Batten disease and MCRI's groundbreaking research currently underway to address this. Learn how you can get involved and aid the team's development of more effective, targeted speech therapies for Batten disease.

Wednesday, 12th July - 8:00-9.00 PM (AEST) **Register [here.](#)**



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.

More information coming soon



Funrun Mania!

City2Surf is being held in Sydney on:

Sunday, 13th August 2023 [Register here](#)

Beach2Beach is being held in Sydney on:

Sunday, 27th August 2023 [Register here](#)

If you are planning to participate in either of these events or just know someone who is, you can now support BDSRA Australia! Simply access our unique pages on the links above when you register or sponsor a friend/team or make a donation. Please share these links widely and encourage your contacts to link their registration with us.

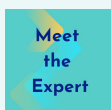
Past



Virtual Coffee catchup - Bereaved Catchup

In the evening of Thursday 4th May, a group of our bereaved parents caught up via zoom from around Australia and NZ. One mum says "I'm so grateful for our community. It's wonderful to stay connected."

If you are interested in attending future catchups, please contact Liz Family Support Coordinator via support@bdsraaustralia.org



Meet The Expert - Complex Care & NDIS Support Coordination in Batten Disease

On Wednesday 3rd May **Jemma Niwha**, Specialist Support Coordinator and Managing Director of Hono Community

Services shared an in depth look into all we need to know about NDIS Coordination of Supports with a wholistic and proactive approach for our complex children and young people. You can watch the recording, [click here](#).



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

On Saturday, 25th March **Dr Joe Vigil**, Psychologist and Batten parent spoke with us live from New Orleans, USA. His knowledge and experience gave us insight in how chronic stress can impact the body and some practical and realistic tips to best avoid carer burnout. There was so much valuable take-home information during this session. To see the recording, [click here](#).



Virtual Coffee catchup - General open-topic Discussion

On Wednesday, 15th March, we had a lively chat covering varying topics - everything from getting to know new people and sharing lived experiences to symptom management and tips for current challenges being faced! Thanks to those who joined us.

Family Support



A new chapter for Fiona in Max's honor

Recently bereaved mum, Fiona Hallum, shares how she's finding her feet after the loss of her son Max. With the support of Carers NSW and Service NSW Women's Return to Work Program, Fiona will be undertaking 100 hours of work placement as a School Learning Support Officer (SLSO) at the school Max attended. "It was confirmation I was doing the right thing; it was just meant to be!" Read more [here](#).

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

Family Register - NEW

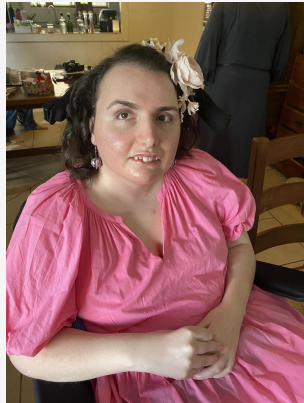
Have you filled in the Family Register form?

Our new Family Register is an important way for



BDSRA Australia to keep families informed of **clinical research opportunities**, and for us to better understand Batten disease in our region and the **needs of our families**. The form takes only 2-3 minutes to complete, and your details are kept STRICTLY CONFIDENTIAL at all times. To fill out the form, [click here](#).

Our New Angels



Georgia

Our Georgia Rose passed away 18/5/23.

Georgia was a warrior and fierce. Her wicked sense of humour will always be remembered fondly. Now she is running wild, seeing, and living her best life. Daughter, sister and granddaughter.



Max

A Champion, Max was 15 years old when he gained his angel wings on 12th March, showing how he often beat the odds as he battled CLN2 Late Infantile Batten Disease. He is remembered for his radiant smile and his strength.

Resources



Web bdsraaustralia.org

NEW RESOURCES on the BDSRA Australia website

The Resources page of our website has recently had an overhaul! Check out all the latest support and educational resources, including a new **Current Clinical Trials search tool**. Check out our [Resources page](#).

You can now also find previous editions of our Newsletters on our updated ['News' dropdown menu](#).



Managing behavioural 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](#).



Connecting carers  to support services

Carer Gateway

Carer Gateway is a free Australia-wide service dedicated to supporting the almost 2.65 million unpaid carers living in Australia. Call Carer Gateway - Monday to Friday, 8am to 5pm or visit the website to find out more.

Phone 1800 422 737

Web www.carergateway.gov.au

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!

One of the things I love most about working with the BDSRA is seeing all the wonderful support we receive from the community firsthand. Whether that be birthday fundraisers, weddings, farewells, amazing community events! Everyone is getting so creative, and we LOVE to see it! These

funds go such a long way in enabling us to achieve our goals, especially when it comes to research and supporting our batten families.

I would love to take this opportunity to say a massive thank you to our regular donors, who never ask for thanks, but we want you to know, never goes unnoticed! Your ongoing generosity is making such a difference.

I would also like to say a special thank you to the students and staff of Stanford Merthyr Infants School and their recent Cake stall fundraising efforts!

This year we have been able to fund another three major research grant projects thanks to all of these incredible contributions!

Amanda Gilpin
Treasurer

Tax Time message

Thinking about giving your hard earned money to a charity close to your heart rather than the tax man? BDSRA Australia is about real outcomes and making a difference, help us turn Hope into a Cure. Donate before June 30th.

[Donate Now](#)

Fundraising

Funrun Thank You!

A special thank you to Sally Brauer who participated in the HBA Run for a Reason held in Perth on Sunday 21st May and through her network raised an amazing **\$2835** using our dedicated BDSRA Australia fundraising page!

For more information on upcoming Funruns, please see the Events section.

ASX Refinitiv Charity Foundation

The news is in and it's fantastic! We have been selected again as one of the benefitting charities for the 2023-24 round of the ASX Refinitiv Charity Foundation program. BDSRA Australia will continue to receive funds from this wonderful organisation and work alongside them supporting their yearly fundraising events.

Last year over \$48,000 was raised from the ASX Refinitiv Charity Foundation's trading day, raffle ticket sales, proceeds from fundraising events and auction items. Thank you to everyone who bought raffle tickets and supported these events.

Our partnership with ASX Refinitiv Charity Foundation would not have been possible without our amazing volunteer Meg Garrido. As she steps away after 6 years volunteering her time on the management committee, we reflect on all she's done raising funds for the benefit of our families and we thank her for her ongoing support.

The next ASX Refinitiv Charity Foundation event is the Racing For Good

Luncheon on **Saturday, 29 July 2023** at Royal Randwick Racecourse. This is a joint venture with the Australian Turf Club which aims to raise money for the 14 charities they support including BDSRA Australia through tickets sales and auctions.

Tickets are \$350 per head or \$3500 per table of 10 and include a 3 course gourmet lunch and a 5 hour beverage package, live entertainment, entry into the racecourse and complimentary in-field parking, dedicated betting facilities, a race guide per guest, raffle prizes and charity auction.

We are also in great need of auction prizes for the day. If you are interested in attending or can donate an auction prize please get in touch with Nikki at info@bdsraaustralia.org or Bobbie at briddle@bdsraaustralia.org


Raising awareness and vital funds

As a not-for-profit organisation, BDSRA Australia relies primarily on fundraising initiatives and philanthropic donations to provide all our programs, services and support. Bringing awareness to Batten Disease can make a difference in our communities from advocating for early diagnosis and tailored supports to raising funds for research and putting Australia on the map for clinical trials and much much more!

Did you see our "Fundraising Update" email on the 17th May? It's one week until **International Batten Disease Awareness Day** when we **Bounce4Batten**, what will you be doing to mark the day? We have glossy flyers, a media release document and email correspondence that you can use to get involved and forward to your own networks.

If you need help or want to talk about ideas, or need any of the above materials posted or emailed to you please contact us, we are here to support you!

Email Nikki at: info@bdsraaustralia.org



The flyer is for International Batten Disease Awareness Day, held on Friday 9th June 2023. It features the BDSRA Australia logo in the top right corner. The main title is 'INTERNATIONAL Batten Disease AWARENESS DAY' in large, colorful letters. Below this, it says 'HELP US BOUNCE TO RAISE AWARENESS FOR INTERNATIONAL BATTEN DISEASE AWARENESS DAY' and 'SHOW YOUR SUPPORT & GET YOUR NETWORKS BOUNCING ON FRIDAY JUNE 9TH!'. The central section is titled 'HOW? - BOUNCE ANYWAY YOU LIKE!' and lists three steps: 1. Take your pic, post on your social media - don't forget to make your post public; 2. Tag us & use the hashtags noted below; 3. If you wish to support our Batten Warriors, please go to www.bdsraaustralia.org/donate. This section is flanked by two images: a woman jumping in a field on the left and a person on a bungee trampoline on the right. Below the list is the heading 'WHY BOUNCE4BATTEN?' followed by a paragraph explaining that bouncing is universal and fun, and that the campaign is about fun and inclusion, shining a light on the serious nature of Batten disease. At the bottom, there is a blue banner with the text 'Tag us on Facebook @bdsraaustralia or Instagram @bdsra_australia Use hashtags #bdsraaustralia #Bounce4Batten #BattenDay2023' and the 'Bounce4Batten' logo on the left.

INTERNATIONAL Batten Disease AWARENESS DAY

FRIDAY 9TH JUNE, 2023

HELP US BOUNCE TO RAISE AWARENESS FOR INTERNATIONAL BATTEN DISEASE AWARENESS DAY

SHOW YOUR SUPPORT & GET YOUR NETWORKS BOUNCING ON FRIDAY JUNE 9TH!

HOW? - BOUNCE ANYWAY YOU LIKE!

1. Take your pic, post on your social media - don't forget to make your post public
2. Tag us & use the hashtags noted below.
3. If you wish to support our Batten Warriors, please go to www.bdsraaustralia.org/donate

WHY BOUNCE4BATTEN?

Bouncing is something universal that kids love & should be able to do. Bouncing creates positive energy, joy & exuberance! The #Bounce4Batten campaign is not just about fun & inclusion, but also shines a light on the serious nature of Batten disease, a condition that robs children of a typical childhood & all the skills & abilities they once had.

Bounce4Batten

Tag us on Facebook @bdsraaustralia or Instagram @bdsra_australia Use hashtags #bdsraaustralia #Bounce4Batten #BattenDay2023

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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Batten Disease Support and Research Association Australia is a registered charity.
Australian Registered Body Number: 073 435 202
Australian Business Number (ABN): 56 056 138 99
Our mailing address is: 74 Mclachlan Ave Shelly Beach NSW 226

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