



BDSRA Australia Newsletter

April 2022

Dear families and friends

BDSRA Australia recently held a brainstorming session to develop its new strategic plan for 2022-23. This plan outlines our purpose (ie. 'why we exist'), the outcomes we seek to achieve and, most importantly, how we are going to achieve those outcomes. Overall, we were very happy with what BDSRA Australia has achieved in the last few years – and we felt it is now time to aim even higher. It was a wonderful day – with some excellent ideas.

A few of our key objectives for 2022-23 include:

- (1) continue increasing Australia's presence on 'the global map' for basic and translational research, and to bring clinical trials to Australia;
- (2) providing even more substantive support for families through 'in person' social events, continuing our extremely successful online 'coffee catchups' and 'meet the expert' webinar series, developing Batten disease specific NDIS resources, providing support to our children transitioning from paediatric to adult care and delivering crisis support packages to families in extreme need;
- (3) increasing the level of interest, specialisation, and awareness about Batten disease in Australia across the medical and allied health professions, including to facilitate earlier diagnosis of our children; and
- (4) continuing to regularly engage with our families and friends, encouraging

them to share their stories through local media to create greater awareness in Australia - and to fundraise for BDSRA Australia to make all of this possible!

If you would like a complete copy of the BDSRA Australia strategic plan for 22/23, please contact Nikki on info@bdsraaustralia.org. If you would like some local fundraising ideas, please contact Bobbie on briddle@bdsraaustralia.org.

On another note, I recently spoke at Victorian parliament on behalf of the Genetic Support Network Victoria about Batten Disease for International Rare Disease Day on 28 February 2022. I told the audience that I would never forget the day my son, Tom, was diagnosed with Late Infantile Batten Disease. I would never forget being told, 'there is no hope' and 'there is nothing we can do'. No parent should ever be told this. No parent should be left feeling that they are alone in this battle. And, who knows? Sometimes by doing something, in the face of 'nothing', you might just achieve a greater result that you could ever imagine.

Have a lovely and safe Easter.

Kate Beattie

Easter 2022

President, BDSRA Australia



Research, Medical and Scientific Affairs

It has been a busy start to 2022 with some exciting clinical trial developments. Several new studies have commenced patient recruitment for CLN3 disease (BBDF-101), CLN2 disease (intraocular cerliponase alfa) and CLN5 disease (gene therapy) respectively. See news links below.

We continue to be 'politely persistent' in advocating for Australian patient access to NCL clinical programs and natural history studies, and maintain regular communication with the industry sponsors and program leads. We will continue posting relevant updates and, in the coming months, plan to host another research update webinar series. However in the meantime, if you would like further information on any specific studies, please reach out to me at research@bdsraaustralia.org

In other news, the 2022 round of our competitive Research Grant Program will be open next month for Expressions of Interest. Each year, BDSRA Australia invests around \$100,000 toward promising research in our ANZ region. For further information head to our website at www.bdsraaustralia/research

We are delighted to offer our families and supporters an opportunity to hear from some of our recent grant recipients at the 'Meet the Researchers' free online event on May 11. This live webinar will showcase some of the brilliant, ground-breaking research being carried out across our region, and will be delivered in simple, easy-to-understand presentations followed by a live discussion and Q&A. Registration details are in our Upcoming Events below. We hope to see you there!

Warm regards,
Dr Ineka Whiteman
Head of Research, Medical and Scientific Affairs

Challenging Childhood Dementia Symposium



Dr Ineka Whiteman spoke on behalf of BDSRA Australia at the inaugural 'Challenging Childhood Dementia Symposium' in Sydney. This was a valuable opportunity to connect and brainstorm with other researchers, clinicians and advocacy groups from around the country.

[Read more...](#)

Recruitment for Phase I/II trial in CLN3 disease now underway

The Beyond Batten Disease Foundation (BBDF) and Theranexus are now actively recruiting patients for its Phase I/II clinical trial. Six patients 17-year-old or older will receive BBDF-101 in escalating doses during the first 4.5 months to establish tolerability and pharmacokinetics of trehalose, miglustat and a combination of trehalose/miglustat.

[Read more...](#)

Brineura being tested on eyes of children with CLN2

Brineura being tested on eyes of children with CLN2. Brineura (cerliponase alfa) is an enzyme replacement therapy for the treatment of CLN2 disease, and is typically infused directly into the...

[Read more...](#)

Clinical trial now enrolling for CLN5 disease gene therapy

We are so excited to share that the clinical trial for the investigational gene therapy for CLN5 disease is now enrolling participants at the trial site, University of Rochester Medical Center in Rochester, NY, USA... This investigational clinical trial, sponsored by Neurogene....

[Read more...](#)

Family Story

Ben & Alex are the older siblings of Emily, diagnosed with CLN3 Juvenile Batten Disease. They have found support through Young Carers.

Ben explains what is most helpful for him "Respite ...and I like getting to know people who have some of the same difficulties"

Alex tells of his experience "Young Carers offer fun camps which include various different activities. The kids attending the camps all care for someone with a disability which can be encouraging and nice to be around someone who understands. It is also nice to get out and do activities that you would not normally do without a group."

Young Carers Network is a Carers Australia initiative, accessible by referral through the national Carer Gateway.

To learn more about how the Young Carers Network are able to support siblings in your family situation and for contact details, use the buttons below to read further.

[Read more...
Sibling Support](#)

[Young Carers
webpage](#)

Below are photos of some activities that Ben & Alex have participated in with Young Carers



Events

'Meet the Expert' in February was a success!

**Meet The Expert -
Education Series 2022**

BDSRA Australia is pleased to announce the first event in our 2022 'Meet The Expert' Education Series.

**Pain Management
in Batten Disease.**

LIVE WEBINAR PRESENTATION
FOLLOWED BY Q&A

Wednesday 9th February,
8pm AEST

Register your interest by emailing
info@bdsraustralia.org to receive the Zoom details.

Featuring Guest speaker
Dr Simon Cohen
Specialist Pain Medicine Physician
& Paediatrician at Monash
Children's Hospital & Monash
Medical Centre, Melbourne

**BATTEN
DISEASE**
Support and Research Association
AUSTRALIA

BDSRA Australia thanks Dr Simon Cohen for giving his time to discuss Pain Management in Batten Disease with our families. This was our first event in our 2022 'Meet the Expert' Educational Series. If you would like a copy of the recording and/or slides, please email research@bdsraustralia.org

[Click to see original post](#)

February 28th was 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.

Right now in Australia, there are more than 50 individuals and their families 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.

Upcoming Events



VIRTUAL COFFEE CATCHUP



MEMORY MAKING



FRIDAY 29th APRIL 2022, 11am AEST

BDSRA Australia would like to invite all Batten families from around Australia & New Zealand to join us for a coffee catch up, where you can listen and share your thoughts on meaningful memory making ideas you and your family would like to explore.

RSVP VIA THE LINK OR EMAIL

info@bdsraaustralia.org for the details



Please use the link below to register for the VIRTUAL COFFEE CATCHUP

<https://events.humanitix.com/coffee-catch-up-making-memories>

Meet The Researchers Education Series 2022



LIVE WEBINAR PRESENTATION FOLLOWED BY Q&A

Hear from our 2021 Research Grant recipients,
who will be presenting the latest research
in easy-to-follow presentations.



**Wednesday 11th May,
4.00 - 5.30pm AEST**

FEATURING GUEST SPEAKERS

A/Prof Anthony Cook

Wicking Dementia Research &
Education Centre, University of Tasmania.
*CLN3 disease stem cell models for drug
screening*

A/Prof Ronald Clark

School of Chemistry, University of Sydney
*Understanding the role of protein-
membrane interactions in CLN3 disease*

Dr Nadia Mitchell

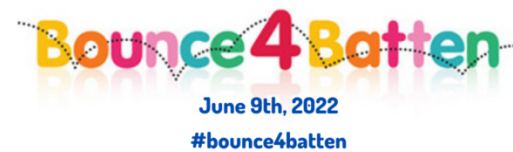
Department of Wine, Food and Molecular
Biosciences, Lincoln University, NZ
*Advancing gene therapies for CLN5 and
CLN6 disease*

Prof Alex Hewitt

Department of Wine, Food & Molecular
Biosciences, Lincoln University, NZ
*Advancing gene therapies for CLN5 and
CLN6 disease*

Please use the link below to register for MEET THE RESEARCHERS

<https://events.humanitix.com/meet-the-researchers-bdsra-australia-education-series-2022>



Each year we ask the community to show their support for International Batten Disease Awareness Day on the 9th June by Bouncing for Battens.

Show your support in just 4 easy steps:

1. **START BOUNCING** any way you like!
2. **SNAP A PHOTO** of someone bouncing.
3. **SPREAD THE WORD** by sharing your photo on social media using #Bounce4Batten#bdsraaustralia
4. **FOLLOW AND TAG US** on Facebook @bdsraaustralia or Instagram @bdsra_australia

Fundraising

ASX Refinitiv Charity Foundation

In February the ASX Refinitiv Foundation held their annual Golf Tournament and Dinner and in March they held their Regatta. BDSRA Australia are privileged to be one of the ten charities supported by this foundation. We had volunteers helping at both events which were amazing as usual.

Save the date - Saturday 30th July for Race Day in Sydney.

Thanks to all who bought tickets in the ASX Refinitiv Charity Foundation Raffle 2021 that was drawn in March 2022. We can confirm that BDSRA Australia has benefited by receiving over \$13,000 from this effort.

The news is in and it's fantastic! We have been selected again as one of the benefitting charities for the 2022-23 round of the ASX Refinitiv Foundation program and will continue receiving funds from this wonderful organisation.

A very special thank you to our Meg Garrido, without whom we would never have received this support.

We would love to hear from you if you are interested in attending or volunteering for BDSRA Australia at the next ASX Refinitiv Charity Foundation event - please email info@bdsraaustralia.org to let us know.



Photo: Meg (far left) and volunteers at the ASX Refinitiv Charity Foundation 2022 sailing regatta

Our New Angel

Harper

Harper Riley Murnane is free from Batten Disease, our beautiful amazing Jitterbug grew his wings on 26th March. Our freckled nose, blue eyed boy packed a very full life time into his 5 years of life, and touched many people. BDSRA Australia & Batten community, send our love to Haidee, Matt, Zana and all the extended family and friends.



If you have lost a child to Batten Disease and would like to connect with other bereaved parents in Australia and New Zealand, via our Bereaved Facebook group or our Bereaved Virtual Coffee Catchups on zoom, please contact Liz at support@bdsraaustralia.org for more information.

Noah's Story - A Batten Disease Journey



This short video is a moving story of one family's devastating journey with Batten disease, told through the eyes of Noah. Our hope is that by sharing stories like this, we can raise awareness, educate and advocate for our Batten families around the world...

[Read more...](#)[Watch Video](#)

Donations

Thank you to all our valued supporters who have recently donated to BDSRA Australia. Our deepest gratitude to our wonderful regular donors. Without your support we would not exist.

You can support BDSRA Australia by giving via our website.

[Donate Now](#)

Other News

A Journey to find hope and a cure

Our board member, Amanda, and family on an extraordinary journey to find hope – and a cure. You have all our love and support See their amazing story here –...

[Read more...](#)

Research News!

A wonderful article on some of the groundbreaking research being carried out by BDSRA Australia research grant recipient, Dr Nadia Mitchell and her team at Lincoln University, NZ. We are...

[Read more...](#)

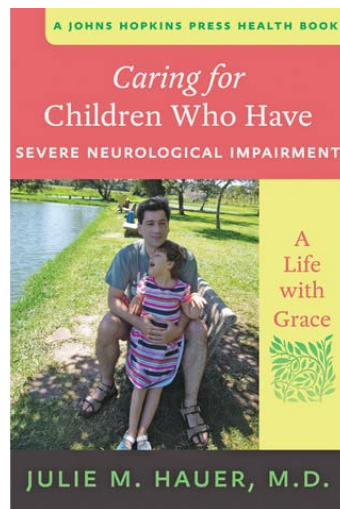
New funding for Childhood Dementia research

RESEARCH NEWS A new \$3 million research grant for research into childhood dementia has been announced by The Hon. Greg Hunt MP. This Medical Research Future Fund (MRFF) grant aims...

[Read more...](#)

Useful Links and Resources

Book Recommendation



Dr Simon Cohen, paediatric pain specialist and guest speaker at February's pain management webinar, has highly recommended this book for our Batten parents and carers. Indeed, we couldn't agree more. A highly informative, practical, holistic and thoughtfully written guide – available for purchase from online bookstores.

Childhood Dementia Support service



Dementia Support Australia (DSA) is here to support our families by providing practical, professional, tailored recommendations and management strategies. The service is free with no referral required. Call the 24/7 service on 1800 699 799 to talk to a trained DSA consultant, or visit DSA Childhood Dementia Support page.

[Read more...](#)

Source Kids

Source Kids is an Australian magazine for parents, carers, families, professionals and teachers working with children with special needs. Source Kids magazine and website covers a range of topics that are relevant to children with disabilities and their families and carers.

Some of our Batten families made it to the The Source Kids Disability Expo in Sydney this March. Source Kids expos are also held in Melbourne, Brisbane and Perth! The two-day expo brings together the latest products, services and technology and is focused on children, youths and young adults living with a disability up to the age of 25 years.

Source Kids have some ideas of things you could try this Easter.

Source Kids
Easter ideas



Happy Easter!

BDSRA Australia sends warm wishes to all our families and community for a safe Easter holiday.

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@bdsraustralia.org.au



Batten Disease Support and Research Association Australia

Batten Disease Support and Research Association Australia is a registered charity.

[View online](#)