SPRING ADDITION

NOVEMBER 2021 - VOL. 23

NEWSLETTER

HUNTINGTON'S DISEASE TASMANIA

Providing a voice for people with Huntington's disease and their families

Available now!

Our 2022 calendar locally designed featuring the finely detailed artwork of Kristy Burgess from our Huntington's community.

To purchase please visit our website.

100% of the proceeds goes to helping Tasmanians with Huntington's Disease

We thank Kristy for her time and contribution spent on this beautiful artwork.

In this issue

From the President's Desk

As the end of 2021 is almost upon us it's time to look back on the year that was.

Whilst the pandemic continued to impact many states across the nation, we have been fortunate in Tasmania to enjoy the freedom of living without lockdowns. This gave us the opportunity to keep providing services and support to our HD community, in a COVID safe manner.

It is with great excitement that I share this watershed moment with the announcement in the recent state budget that our organization's request for $80,000 per annum funding to support an Executive Officer plus admin support has been granted. Due to the vision and deep commitment of those who pioneered the organisation 44 years ago and all who have since worked tireless in a volunteer capacity, this is a win for you. Moving forward, this money will allow our organization to continue to support Tasmanian people with HD to live their best possible lives. A special thank you to all who have contributed to this wonderful result. Recruitment is underway.

2021 was a busy year for the organization. Important events/priorities included:

- Advocating on behalf of our community remained a high priority.
- Our non-clinical counselling, and 24-hour phone service has continued.
- Education remains a top priority with regular sessions provided to clients, families, carers, and the community.
- A consortium of other HD associations from around Australia that was convened last year has continued to work collaboratively, informally sharing resources, information, expertise and creating a strong voice for all impacted with HD across the nation. A Memorandum of Understanding has been signed by us and Chair's from Queensland, New South Wales/ACT, South Australia/NT, and Western Australia.
- The HD National Conference was held virtually this year and hosted by NSW. It was an outstanding success with international and local presenters attracting over 400 registrations. A big thank you to Julie Burgess, one of our Tasmanian family members who gave a passionate presentation.
- As part of National Consortium, we continue to focus on YPIRAC (Young People In Residential Aged Care),advocating that people with HD should be able to choose where and how they live and be supported within reason to meet their lifestyle and accommodation choices in line with the Objects of the NDIS Act and the UN Convention on the Rights of Persons with Disabilities. This includes the right to live alone.
- Launceston Tuesday bus groups continues, participants enjoy their outings and friendship. Hopefully we can expand this to more groups next year. Monthly Southern support group commenced in Hobart. The group will continue six weekly
- providing connection to those with HD, family, carers and other support services. Contact Aleks on 0458 450 705.
- All most twelve months of planning we are now ready to commence our Youth and Young Adults Peer Support Service (YaYAPSS). See details further in Newsletter.
- Our wonderful support person Barbara continues to knit Beanies for us to sell. Following our letter to "Macca" on "Australia all over" earlier this we had orders from ever State and Territory.

As this challenging year heads towards its close, surely 2022 will be kinder, more stable and perhaps, even COVID free!!!! This will be the last newsletter for 2021 and I would like to take this opportunity to wish you, your families and friends ***a safe, happy and wonderful Christmas*** and a ***new year filled with love, health, and happiness.***

To those who have lost loved ones this year I hope you are able to find grace and peace this Christmas.

Warmest Regards

Pam Cummings

Vice President Report

It gives me great satisfaction to let the Tasmanian HD Community know that we have commenced a Southern Support Group based out of Hobart. HD Support has been somewhat inactive in the southern half of the state for quite a while due to a number of factors.

On the 19th of September, the Southern Support Group had its first public meeting, held at Harbour Lights Café on the Hobart Waterfront. The meeting was attended by around 8-10 people and was a great opportunity to meet new faces and share in healthy discussions across a broad range of topics!

Our next meeting will be held on;
Sunday 12th December 10am
Bear with Me Café – 399 Macquarie St South Hobart

Everyone is welcome to attend, minors to be accompanied by an adult, you are more than welcome to purchase your own food and drink during the meeting.
Look forward to catching up, New members welcome!!

Another year is almost over, and we recently held our AGM for 2021. I would personally like to pass on my appreciation to Pam Cummings for the never-ending amount of time and effort that she puts into supporting the HD families across Tasmania.
Its exciting that Wendy Weeks is continuing on in the role of Secretary – your work is also greatly appreciated!
Congratulations to our New Board members – Kayla, Michael & Linda. Welcome to the team, I have no doubt that moving forward, your input and enthusiasm will help drive the organisation forward.
And, of course, thanks to our existing board members for your continued support and efforts over the last 12-month period. It has been a challenging 12 months, but it has been fantastic seeing everyone adapt and come together as required.

I look forward to working with the organisation, and hope to help drive us forward into the next chapter of supporting Tasmanian families with Huntington's.

Aleks Long

Huntington's Disease Tasmania
Elected Board Members 2021/2022

President - Pam Cummings
Vice President - Aleks Long
Secretary - Wendy Weeks
Treasurer - vacant
Public Officer - Paige Dale
Board Members - Kayla Palombo, Michael Frankland & Linda Nichols

Condolences

We send our loving thoughts to the Russell family on the recent death of Ray.

Huntington's Disease Southern Support Group

Next Meeting

When: **Sunday 12th of December 2021**
Where: Bear with Me Café
399 Macquarie Street, South Hobart
Time: 10am - 11am

This group is open to anyone with a connection to Huntington's Disease.
Those with HD, family, carers and other support services. Come and have a chat, hang out, ask questions and connect with our HD community.

This group welcomes all ages, however minors should be accompanied by an adult for the initial meeting.

For further information please call Alex on 0458 450 705

Proudly Supported by Huntington's Disease Tasmania

Introducing Kayla

My name is Kayla Palombo and I was recently elected to the board for the Huntington's Disease Association of Tasmania. I'm really excited to be appointed to this position as a person who is gene positive to this disease but not symptomatic. I have a lot to offer this community with a background in social justice state government work in the criminal justice system and project management. With a strong background in governance, policy, risk management, stakeholder engagement, connecting non-government and government agencies and delivering therapy to people within the criminal justice system I'm excited to bring these skills to the Association. I personally have a love for animals in particular chickens and dogs, I love spending time outdoors and computer gaming. Prior to moving to Tasmania in May last year I had been engaged with the Huntington's Association of Queensland. Work I'm currently involved in includes being a representative of the LGBT IQ diversity and inclusion work at the Department of Justice and I'm hoping to also be involved in the disability space at the Department of Justice. My current project with the Association is the youth program which is about to be rolled out in 2022.

I'm looking forward to serving this community and hope to speak with many of you.

YaYAPSS

Youth and Young Adult Peer Support Service
Provided by the Huntington's Disease Association of Tasmania

Huntington's Disease Tasmania is about to launch a new youth program.
Are you a youth, teen or young adult with family who have Huntington's Disease? Are you at risk of developing Huntington's Disease? Then this may be the program just for you.

The youth group is looking to work with people aged between 13 and 30 in Tasmania. It will be run on a monthly basis with both in-person sessions and a social media group. We are looking to meet with people who may be interested in this group to learn more about what's on offer and who might like to sign up.

We will be holding online information sessions on Saturday, 11 December and Saturday, 15 January 2022. Come and join our zoom meeting to get a better understanding of the program and to meet the facilitator Kayla

Where can I learn more? Come along to one of our information sessions or talk directly with those who run the program: Kayla and Mike on 0412 142 520

Introducing Michael

Hi I'm Michael, my partner Kayla and I are new imports to Tasmania having moved to Hobart from Brisbane in May 2020. We visited Tassie for the first time for a brief long weekend holiday in Jan 2020, immediately fell in love with the people, the climate and beautiful scenery and made a snap decision to move here only a few months later. It turned out to be the best decision we've ever made, and we are currently living our best life in the Huon Valley spending all our free time bustling around in the garden, playing with our dog and chatting to our chickens. We are pleased to be able contribute to Huntington's Tasmania as we ourselves deal with the reality of battling Huntington's and see firsthand the difficulties faced by those at all stages of living with this disease.

I have several years' experience working with associations and membership bodies and am currently the State Manager for the Australian Computer Society here in Tasmania and so I hope I can bring some of that knowledge and experience to Huntington's Tasmania as a member of the board to help drive some great outcomes for other families going through the same things as we are.

I'm looking forward to meeting you in the near future, feel free to reach out any time.

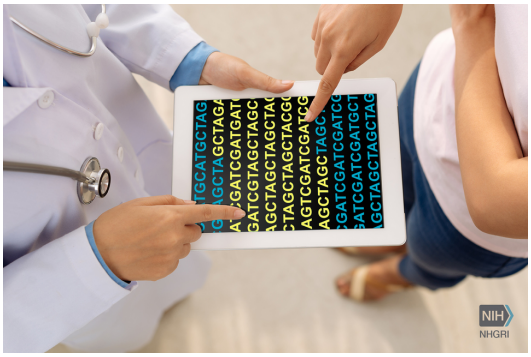
Updates from the EHDN Meeting 2021

Last month, HDBuzz attended the online European Huntington's Disease Network (EHDN) meeting. Read our summary of all the latest clinical trial updates. By Dr Rachel Harding October 28, 2021 Edited by Dr Jeff Carroll

Last month, the Huntington's disease (HD) research community, patients and other stakeholders met online at the European Huntington's disease network (EHDN) conference. Despite the ongoing global pandemic, there is a tremendous amount of work underway in labs and clinics around the world as researchers continue to better understand HD and how we might best treat it. Although 2021 has been a year of disappointing news in some respects for the HD patient community, there are lots of reasons to be hopeful as we learn from past clinical trials and use that information to inform new ideas for medicines which we are now beginning to test in the lab and the clinic. Here, we give an overview of the clinical trial updates.

Scott Schobel from **Roche** gave an update on the huntingtin-lowering drug tominersen and the GENERATION-HD1 clinical trial. The decision to halt dosing in this trial was made earlier this year following advice from the Independent Data Monitoring Committee (iDMC), a neutral organisation whose job it is to review the data at set time points throughout the trial. Following this announcement, more than 40,000 samples needed to be shipped from all around world to designated labs for analysis. The samples need to be analysed using predefined procedures and in relatively few batches to ensure the data obtained from the samples is of the highest quality. Having good quality data from the trial should help scientists better analyse exactly what happened. This is a huge logistical operation, but analysis of the data is now underway and Roche hope to begin sharing some of their findings by the end of this year with the full analysis being released early next year. This might seem slow and frustrating, but Roche want to avoid releasing partial information which might lead to misinformation or unrealistic expectations which would be extremely unfair to the HD patient community. Many open questions remain; does the stage of HD a patient is at play a role in how well tominersen might work? Was too much drug administered in the trial? Which factors about a patient predict response to drug? Hopefully we will have answers to these questions soon. If you want to know more about the halting of GENERATION-HD1, we recently wrote a detailed Q and A with Roche about the halting of this trial and next steps for Roche on HDBuzz.

Maurice Zauderer from Vaccinex gave an update about pepinemab which was investigated in the SIGNAL trial. Pepinemab is an antibody therapy which specifically targets a protein in our bodies call SEMA4D. SEMA4D has an important job in inflammation, a response that is critical to how our immune system deals with infections and some diseases. Last year, we reported that unfortunately pepinemab did not improve symptoms in HD patients and that the trial did not meet its primary end points – clinical goals decided upon before the trial begins. However, since last year's announcement, scientists involved in the trial have been re-analysing the data to see if they might eke out some additional information on the usefulness of pepinemab. In this "post-hoc" analysis, it seems that pepinemab might be beneficial in early-stage HD patients for improving certain behaviours such as apathy. However, it is important to remember that this Is not what the trial was designed to work out so these findings should be treated with caution.



Even though the COVID-19 pandemic has disrupted so much of our lives, scientists and clinicians have still been extremely busy with their research. At EHDN we heard lots of updates and discussion about the different Huntington's disease clinical programs underway or about to start
Image credit: Darryl Leja, NHGRI

Vissia Viglietta from Wave Life Sciences gave an update on the latest trial they are working on which will test WVE-003 in a trial called SELECT-HD. Wave's approach is to selectively lower the toxic form of the huntingtin protein whilst preserving the healthy huntingtin protein. This rationale is based on lots of data which shows that normal huntingtin protein is very important for brain function, especially in the context of different stresses. Wave can achieve this selectivity by targeting a specific signature in the DNA code which is only found in the toxic huntingtin gene. Although Wave's previous clinical trials had disappointing results, they are optimistic that this new drug will perform better as they have changed the chemical decorations on the drug which should make it more potent, last longer in the body and spread more easily to the regions of the brain it needs to be. They have been doing lots of work in the lab, testing this latest version of their drug in cells in a dish, mice, and monkeys, all of which have had encouraging results.

David Cooper from Uniqure gave an update on their gene therapy approach to treating HD, currently underway in trials called HD-GeneTRX-1 and HD-GeneTRX-2. Uniqure's drug, called AMT-130, is a one-shot treatment delivered by brain surgery. AMT-130 provides your body the recipe to make the therapy which will lower the levels of the huntingtin protein. Uniqure have been busy with lots of proof-of-concept experiments in different HD models. They have tested AMT-130 in cells in a dish, HD mice and rats, as well as bigger animals like monkeys. Uniqure have also tested their drug in HD pigs where they have tested long-term effects of treatment – an important experiment as this treatment is an irreversible procedure. To date, Uniqure's data suggest that the drug is safe long-term in these animal models. The HD-GeneTRX-1 trial aims to test safety of AMT-130 in humans, how long the drug stays in the body as well as how AMT-130 affects different markers of HD progression. The trial will enroll 26 early-stage HD patients at 12 different HD study centres in the US and will run for 1 year with follow up for a further 5 years. Because the drug is delivered by brain surgery, the brain anatomy of each potential participate is evaluated to ensure they are good candidates for the trial. A similar study, HD-GeneTRX-2, will run in Europe with 15 participants across 3 different sites.

Irina Antonijevic from Triplet Therapeutics gave an update on their drug TTX-3360. Triplet's approach to treating HD is to lower the levels of a key DNA repair protein identified in HD patients. In previous studies, scientists have searched for genetic traits that influence the age at which someone with the HD mutation first experiences symptoms. We have known for a long time now that patients with the same CAG number can have symptoms start at very different ages so scientists suspected other genes might be "modifying" the age of onset. Triplet's target gene, MSH3, was identified as one of these "modifying" genes. Triplet's drug will lower the levels of MSH3, with the aim of delaying onset of disease symptoms on HD patients. Lowering the levels of MSH3 is safe in mouse and monkey models so Triplet are hopeful that the drug will also be safe in humans. Getting the drug into the right parts of the human brain is challenging so Triplet have decided to opt for a novel delivery approach for their drug, compared to other ASO drugs tested in HD so far. This approach will allow an implanted catheter to deliver Triplet's drug to the deep brain structures we think are important for HD symptoms. Triplet hope to start their clinical trial for TTX-3360 next year so watch this space!

Michael Hayden from Prilenia gave an update on their drug, pridopidine. Pridopidine works by targeting a protein called the signal-1 receptor (S1R) which has been shown to improve signs of HD in different models in the lab. An advantage of pridopidine is that it may be taken as a pill – not surgery or spinal tap. However, the previous PRIDE-HD clinical trial which tested pridopidine in HD patients did not improve patient movement symptoms. There were some glimmers of hope however that some symptoms of HD, also referred to as total functional capacity (TFC), might be improved following pridopidine treatment so now Prilenia is running PROOF-HD. This study will test more people (480 participants) for much longer to see if this feature of HD is improved.

Beth Borowsky from Novartis gave an update on their drug, branaplam. Branaplam can switch different genes on or off and has been shown to lower huntingtin levels. Branaplam can be taken as a pill so it places significantly less burden on patients than spinal tap or brain surgery approaches to huntingtin lowering and will also treat the whole body, not just brain and nerve cells. Novartis have shown branaplam works well in the brains of HD mouse models to lower the levels of HTT. Novartis also have a lot of data from SMA patients treated with branaplam which shows that the drug is safe and well tolerated as well as also lowering the levels of HTT in the blood of these patients. However, SMA patients are children, so Novartis is conducting a “first-in-adult” clinical trial, treating 32 healthy adults with branaplam to check safety and work out an appropriate dose of the drug to give to adults. This study informed design of a Phase IIb trial where branaplam will be tested in early-stage HD patients. Recruitment for this trial will begin at the end of 2021 in sites across Europe and North America.

8 different drug discovery companies presented at EHDN on their approaches to treat Huntington’s disease. Maybe one day, one of these drugs might be a new medicine to treat people with HD.

Brian Pfister from PTC Therapeutics gave an update on their drug, PTC518 HD. PTC518 is another drug which may be taken as a pill to lower the levels of the huntingtin protein recipe molecule, like branaplam. PTC have shown that their drug lowers the levels of huntingtin in both the blood and brain of HD mouse models. PTC518 is able to lower huntingtin across lots of different brain regions in these mice which indicates the drug spreads well. In studies with monkeys, PTC have shown that their drug is able to cross the blood-brain barrier, again, demonstrating that PTC518 should be able to reach the important regions of the brain after being taken as a pill. There is currently an early stage trial underway for PTC518 testing safety of this drug in healthy participants. Importantly, data from this clinical trial shows that the more drug given to participants, the more the levels of the huntingtin are lowered by. Unlike gene therapy approaches, PTC’s huntingtin lowering is reversible so if you stop treatment, huntingtin levels should bounce back to normal. Later this year, PTC518 will enter a Phase II clinical trial so hopefully we will have some more news for you soon.

It’s exciting to see so many companies continue to work on a diverse range of approaches to treat the route cause and symptoms of HD. We look forward to reporting on more updates soon as many of these trials get underway and start reporting their findings.

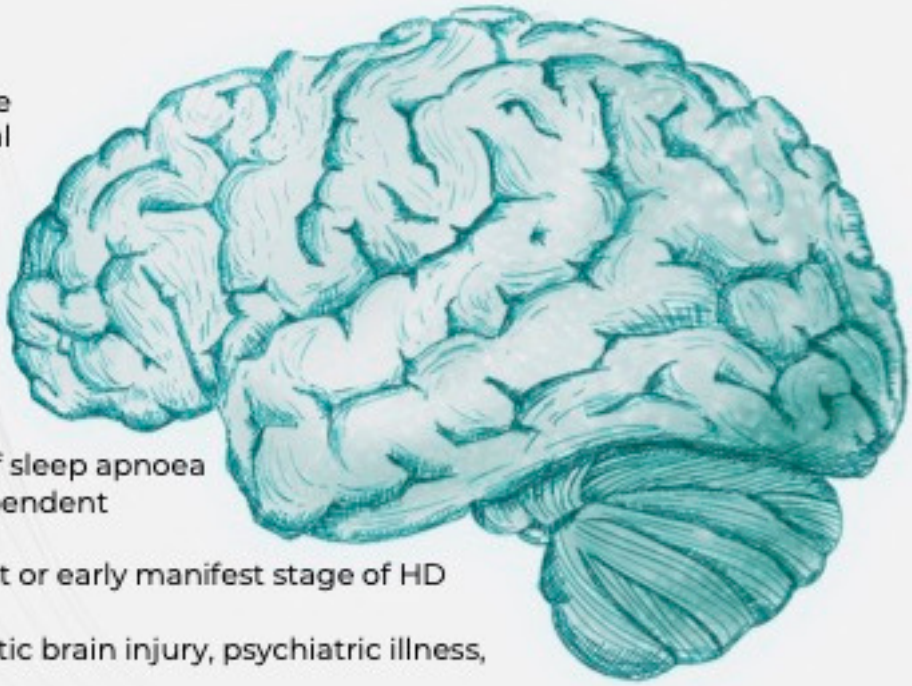
Jeff Carroll is on the Scientific Advisory Board of Triplet Therapeutics. He has conducted sponsored research with Triplet Therapeutics and Wave Life Sciences. No one from Wave or Triplet had any input to this article. For more information about our disclosure policy see our FAQ... online

SLEEP AND GUT HEALTH IN HUNTINGTON'S DISEASE

We are looking for people with and without Huntington's disease (HD) to take part in a study investigating sleep quality and the gut.

Who are we?

This study is being led by Dr Yifat Glikmann-Johnston, Professor Julie Stout, and Emily Fitzgerald (Clinical Neuropsychology PhD candidate) from the Stout Lab at Monash University.



You may be eligible if:

- You're aged 18-65
- You're not a shift worker
- You do not have a diagnosis of sleep apnoea
- You're not drug or alcohol dependent
- You do not have HD
- You are within the premanifest or early manifest stage of HD
- You are gene negative for HD
- You have no history of traumatic brain injury, psychiatric illness, or learning disability
- You have no diagnosis of irritable bowel syndrome, coeliac disease, Crohn's disease or diabetes
- You have not travelled across three zones in the last month

What will I need to do?

- Provide a stool sample to assess gut function
- Wear an activity monitor, like a FitBit, and complete a sleep diary every day for 14 days
- Complete a set of online questionnaires
- Complete a set of cognitive tasks via mobile and telehealth
- This study will be conducted from your home, which means you don't need to travel anywhere to participate!

Reimbursement

You will be reimbursed up to $120 upon completion of the study

MONASH University

For more information contact: Meg Rankin (03) 9903 4695
Med-HDsleepgutstudy@monash.edu

MUHREC Project IDs:
27008 & 23253

HD Happenings

We are looking for photos and stories to be included into our newsletter. It is a great opportunity for families to get involved and share with other HD families their photos and stories. Whether you're affected by HD yourself, a carer or family member we would love to hear from you.

Christmas & New Year Closure

Burnie Office Closure

Our office will be closed from December 18th 2021 until January 10th 2022.
24 hour phone support will be available.
If you need help or support during this time please contact 0417 309 818

HUNTINGTON'S DISEASE TASMANIA

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