

NEWSLETTER

HUNTINGTON'S DISEASE TASMANIA

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

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Big Penguin, Penguin Tasmania

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From the President's Desk

Hope you are enjoying this lovely Autumn weather and the beautiful colour of the changing foliage.

COVID-19 vaccinations appear to be the hot topic at the moment with many hesitating and uncertain whether to have the vaccine or not. If you have any concerns talk to your Doctor.

I would like to thank everyone who cut and pasted the message I sent to the Premier for consideration when preparing his budget.

The past quarter have been one of positive outcomes as we continue work with other State and Territories.

One of the many joint submissions that we have been part of recently is the one of most concern to our HD families and that is the Joint Submission to the Standing Committee on the NDIS inquiry into independent Assessment under the NDIS. See more later in the Newsletter.



March saw the sad news re the cancellation of both the Roche and Wave trials. Whilst Tasmania did not have direct participation in these trials, we all felt the same devastation and helplessness that was, and still is, in the Research and HD communities across the world. All is not lost as the Data will be useful for future trials.

Most importantly, we would like to acknowledge the very real contribution and commitment of the families who are participating in these studies, as well as the broader Huntington's community for their collaboration. There are still many efforts ongoing – both within the Roche Group and other companies, it remains a hopeful time for the HD community.

HD patients who volunteer in these early trials are heroes to us all.

Please do not forget to register for our National conference, one of our own Tassie family members will be a guest speaker. She is a wonderful lady who will do us proud.

I leave you with this message "Love is the best medicine."

Kindest Regards

Pam Cummings.



Condolences

We send our thoughts and love to the families of Ray Davis, Tammy Seabourn and Corey Eaves who sadly passed away recently.



Huntington's Australian National Conference 2021

Huntington's National Australian Conference as a series of virtual events from 25 May – 1 June 2021.

About this Event

Huntington's NSW ACT is very pleased to announce it will be hosting the next national HD conference as a series of virtual events from 25 May – 1 June 2021. This conference was originally scheduled for November 2020 but was postponed due to COVID-19. Now it makes sense to go virtual as we have no idea when a real event might again be possible.

As a result of being virtual, we can bring you many more international speakers than we could afford to fly to Sydney for a live event, so we hope this is a small compensation for the mostly later in the day timing of sessions and for not all meeting physically together.

Experience has shown that 2-hour sessions online with lots of time for questions are more digestible than sitting in front of a computer screen all day. Sessions will also be recorded so once you have registered, you can participate in your own time or re-visit them whenever you want.

We have decided to focus on four key themes:

- Achieving quality of care
- Achieving better policy outcomes
- Achieving better consumer outcomes
- Research update

Many thanks to Roche for its generous support and also to the Commonwealth Government's National Disability Conference Initiative for its grant. Also thanks to Novacorr for being an exhibitor.



To register visit [www.eventbrite.com.au huntingtons-australian-national-conference-2021-tickets-143020571379](http://www.eventbrite.com.au/huntingtons-australian-national-conference-2021-tickets-143020571379)



Would you like to shape research on housing and services for people with Huntington's disease?

Researchers at La Trobe University and the Summer Foundation are looking for people to help shape a research project. This research project is in partnership with Huntington's Disease Association Tasmania.

The research project is trying to understand the best housing and service options for people with Huntington's disease. We would like people with Huntington's disease or people who are carers to help design and shape this project. We are looking for five to seven people to be part of this group.

If you choose to be involved, you will be asked to attend three virtual meetings from February - June 2021. Each meeting will be 90 minutes and you will be given a voucher worth \$60 each time we have a meeting to thank you for your contribution.

If you would like more information about the study and the community group, please contact:

- Emily Gosden - Kaye by email on emily.gosdenkaye@summerfoundation.org.au - or email Elise Davis on elise.davis@summerfoundation.org.au or phone on 0436 345 578.

HD BUZZ

Day 3

Huntington's disease therapeutics conference 2021 - Day 3

Catch up on all of the latest updates from day 3 of the 2021 CHDI Huntington's disease therapeutics conference #HDTC2021

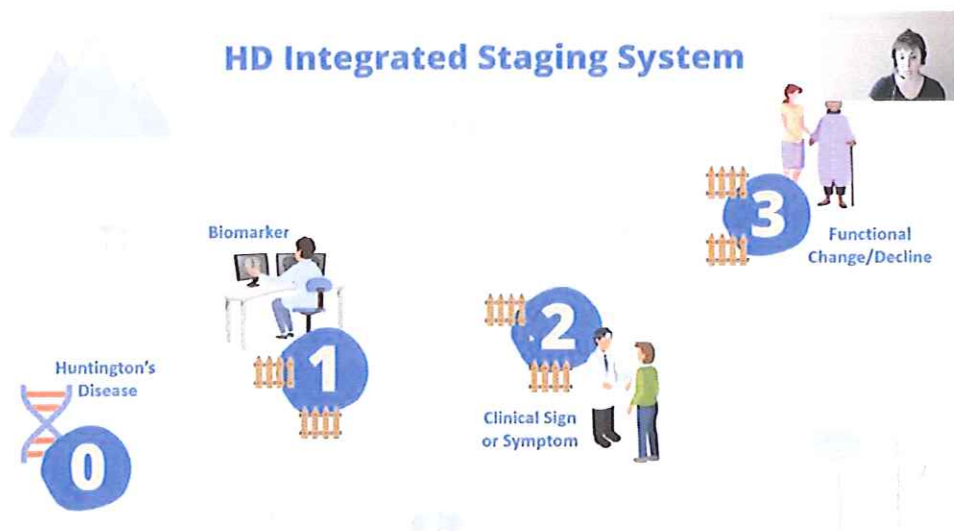


Ed Wild By Dr Rachel Harding, Dr Leora Fox, and Professor Ed Wild
April 29, 2021 Edited by Dr Rachel Harding

We are back for the last day of the virtual 2021 CHDI Therapeutics conference. This article summarises our live Twitter updates on the exciting science being presented, which you can find with the hashtag #HDTC2021. The final session of the conference will provide the latest news on more Huntington's disease clinical programs.

A new way to define the different stages of HD

The first talk of the day was from Sarah Tabrizi and Jeff Long who presented the HD Integrated Staging System (HD-ISS), which aims to redefine the different stages of HD to encompass all the complexities and variability of HD progression.



HD-ISS conceptualises HD as 4 changes happening in a sequence: The disease (the lifelong effect of the gene); being able to detect the gene's effects (biomarkers); symptoms; and functional change (loss of ability to do stuff). This is defined as stages 0 to 3.

The new system is a result of lots of hard work by the HD Regulatory Science consortium, a global network of academics, industry scientists and experts in drug regulation. The HD-RSC exists to put in place a framework that lets good drugs for HD get tested and approved smoothly.

The main aim of HD-ISS is to enable clinical trials to include people before they develop movement problems from HD (motor diagnosis). These trials would aim to prevent or delay HD onset. Tabrizi pointed out that the HD-ISS framework is not a reinvention of clinical diagnostic or staging criteria - it is purely a research tool to allow selection and monitoring of patients for clinical trials - an important distinction.

Current trials use staging systems based on "landmarks" with cutoff values. The most obvious in HD is the concept of "motor diagnosis" which is what was used to diagnose HD even before we knew the genetic cause. But biologically, we now know HD is a continuum - it develops slowly, affects people very differently, and has a long period where people feel and look normal, but we can show with things like blood tests and scans that the mutation has had some effect on the brain.

For research purposes, Tabrizi proposes a new definition of HD. She conceptualises HD as 4 changes happening in a sequence: The disease (the lifelong effect of the gene); being able to detect the gene's effects (biomarkers); symptoms; and functional change (loss of ability to do stuff).

These changes are expressed as stages 0 to 3 and the transition from one stage to another is defined by agreed landmarks. Jeff Long explained that the cutoff values for each landmark were determined systematically from large datasets, for example, monitoring the volume of the caudate lobe of the brain as measured by MRI. Crunching the numbers lets Long plot trajectories showing how a person might be expected to move through the stages during their lifetime.

Tabrizi explained that this kind of modelling lets drug developers plan trials because terminology will be unified including the possibility of intervening before motor symptoms emerge. Importantly, HD-RSC sought input from non-scientist HD family members via the HD-COPE network.

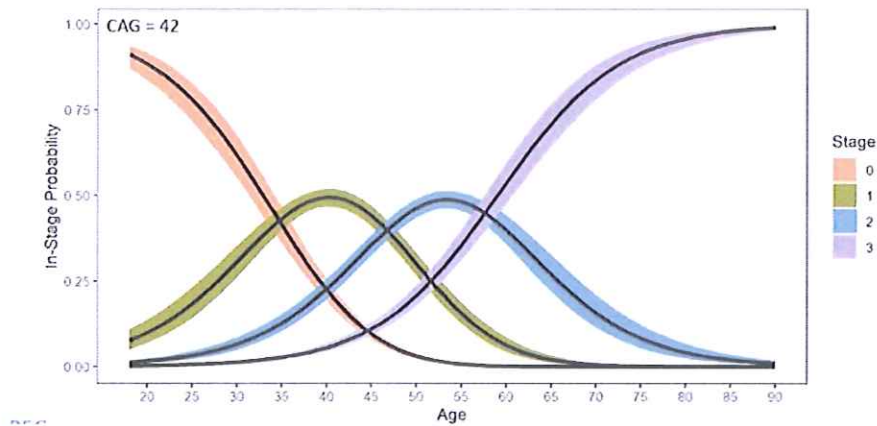
Updates on the huntingtin-lowering trial HD-GeneTRX1 from uniQure

The next speaker was David Cooper who gave updates on the uniQure huntingtin-lowering gene therapy trial, testing the drug AMT-130 which we recently wrote about.

AMT-130 is the first gene therapy for HD. It is packaged inside a harmless virus and delivered to the brain via a one-time surgery. Once there it acts to inactivate the recipe for huntingtin protein. Cooper told us about how AMT-130 was tested in large and small animals and was shown to be safe and well distributed to important regions of the brain.

Next, Cooper introduced the Phase I/II study, called HD-GeneTRX1. It involves 26 people who will get one of two dose levels of AMT-130, or will undergo an imitation surgery. They will be followed closely for a year and more infrequently for up to 5 years. This is a safety study so the main objective is to make sure patients do not experience any dangerous side effects.

Participants are between 25-65 and have early HD symptoms. They also need to have a CAG repeat of 44 or more, and to meet other criteria regarding the size of certain brain areas so that the procedure can be performed safely. The criteria evolved a bit as they screened patients and learned from the first surgeries. 10 patients have now been treated by brain surgery which is performed in an MRI. This is cutting-edge science and has provided valuable lessons learned to the neuroscience community thanks to the selfless volunteers in this study. There are nine sites recruiting patients in the USA and uniQure will be starting a small study in Europe later this year, in which everyone will receive the drug (known as an open-label trial).



The new HD-ISS model allows scientists to predict trajectories showing how a person might be expected to move through the stages 0 to 3 during their lifetime. Here is an example of the probability graphs for someone with 42 CAG repeats.

In the Q&A, Cooper was asked whether the Roche trial results changed uniQure's plans for this trial. He explained that right now they are moving forward as planned, and that their surgical delivery method is different from that used by Roche.

Towards a more collaborative HD research community

The final speaker of the conference was Aled Edwards of the Structural Genomics Consortium, who spoke about how the HD scientific community and in particular industry researchers might better collaborate at the level of drug discovery, the process of identifying genes and pathways to target with therapeutics. Drug discovery often starts with a huge array of possibilities that get narrowed down over time as research reveals more about the biology of HD.

Edwards is a huge proponent of open science and data sharing, and he suggested that the perceived legal and financial barriers to doing this, even within the private sector, can be easily overcome and could benefit individuals with HD and many other diseases. The HD community is already so tightly collaborative that it could potentially set the bar for others by implementing some of these strategies. Excellent food for thought at the end of three packed days of sharing the latest in HD research amongst academics, clinicians, and members of industry.

Looking forward to HDTC 2022 already!

And that's all folks! We hope this has been a helpful way for everyone to learn about all of the latest news in the pursuit of medicines to treat HD.

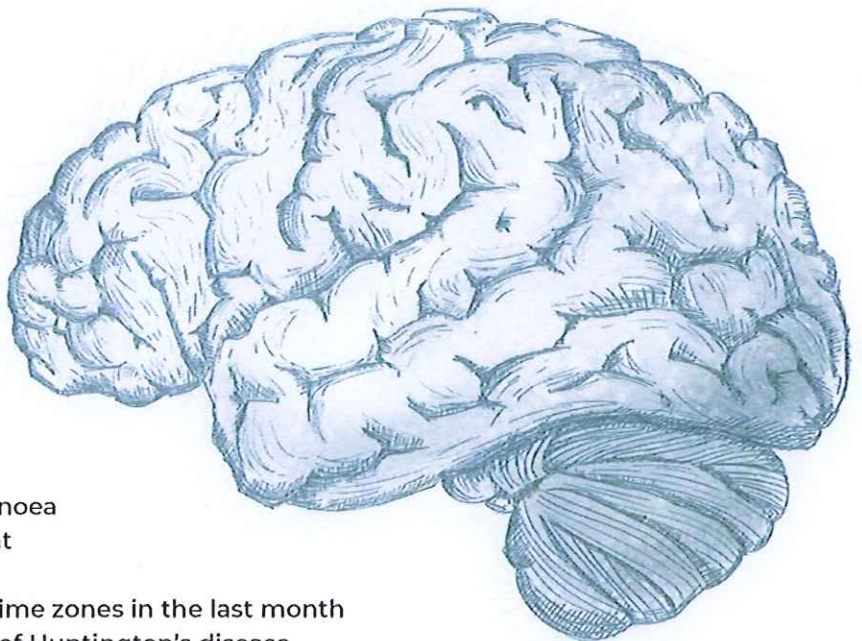
Rachel Harding has no conflicts to declare. Ed Wild is a close colleague of Sarah Tabrizi. Leora Fox works for the Huntington's Disease Society of America, which holds nondisclosure agreements with uniQure and Vaccinex, mentioned in this article.

SLEEP IN HUNTINGTON'S DISEASE

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

Who are we?

This study is being undertaken
by Emily Fitzgerald
(Clinical Neuropsychology PhD)
under the supervision of:
Professor Julie Stout
Dr. Yifat Glikmann-Johnston
Associate Professor Clare Anderson
Dr Melinda Jackson



You may be eligible if

- You're not a shift worker
- You don't have diagnosis of sleep apnoea
- You're not drug or alcohol dependent
- You're aged 18-65
- You have not travelled across three time zones in the last month
- You have or do not have a diagnosis of Huntington's disease
- You have no history of traumatic brain injury, psychiatric illness or learning disability

What will I need to do?

- 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
upon completion of the study **\$80**



For more information contact: Emily Fitzgerald (03) 9905 1918
med-HDsleeeppgutstudy@monash.edu

MUREH Project ID: 23253

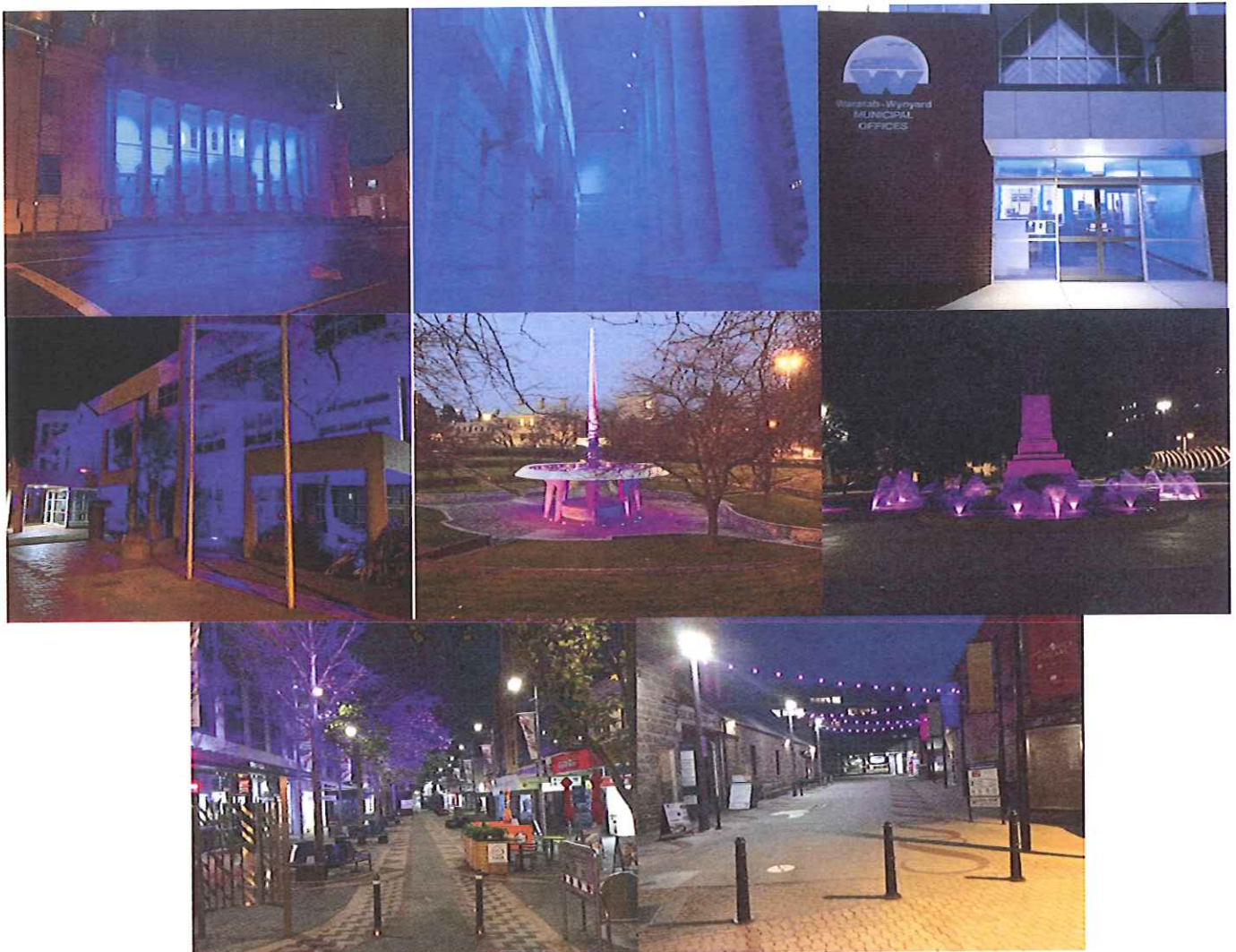
LIGHT IT UP 4 HD!

Once again this year we have joined with the Global Community with our Light up to bring Awareness to HD.

This year we are thankful to

- Wynyard City Council Chambers (All of May)
- Burnie City Council Chambers (All of May)
- Launceston City Council Chambers 1-6 th
 - Hobart 10-13 th
- Paranaple Centre Devonport
- Big Penguin, dressed in HD Tas colours

If you have any ideas for next year's Light up we would love to hear from you.



Peanut Butter Hot Chocolate



As the weather cools down, try out this new version of Hot Chocolate! A melding of flavours gives the drink a number of layers of chocolate, peanut butter or a mix of the two. If doing the vegan version and using almond milk, there is a third flavour to enjoy.

Ingredients

- 50ml water
 - 3 squares of chocolate. Any plain is good, dark makes it a little bitter.
 - 1 heaped tablespoon of peanut butter (smooth is best but crunchy works)
 - 150ml milk (for vegans, go almond, and there is a taste extension right there...)
-

Peanut Butter Hot Chocolate

Method

- Pop the water, peanut butter and chocolate together in a small saucepan on medium heat.
 - Stir well until the chocolate and peanut butter have melted and formed a glossy, sticky paste.
 - Add a bit of the milk and stir it in, add more of the milk and stir, then finally all of it.
 - Do NOT pour all the milk in at once at the beginning, as it will seize and go yuk.
-

To Serve

Pour the milky mix into your mug and drink up slowly and with the sort of reverence you see in advertisements.

Microwave Version

You can make this in the microwave in *twenty-second* bursts.

- Into a microwave-safe bowl place the water, chocolate and peanut butter.
 - Twenty seconds in, then stir well, then twenty seconds again and stir. Continue until the mixture is melted and combined.
 - However, just like porridge, it's not as good and creamy as when gently coaxed along on the stove.
-

Enjoy with a biscuit or a piece of slice.

Sharing

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.

Can you Help?

Leanne, who volunteers in our office would like to compile a Recipe book as a fundraiser.

Please send your favourite Recipe /Hints and Tips to her via our office.

I am sure there are wonderful cooks out there who could contribute.



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