



Huntington's disease research news.

In plain language. Written by scientists.

For the global HD community.

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Oz Buzz Updates: Day 2

Day 2 of our coverage of the Huntington's disease World Congress 2011 in Melbourne



By [Dr Ed Wild](#) September 13, 2011 Edited by [Dr Jeff Carroll](#)

Our second daily report from the Huntington's disease World Congress brings together all the live updates from our twitter feed. Video of the day's Oz Buzz session - with news, interviews and features - will be available to watch at HDBuzz.net later this week.

Tuesday, September 13, 2011

8:44 - G'day from Melbourne: day 2 of the Huntington's Disease World Congress. Video of yesterday's live Oz Buzz session is now on youtube.com/user/hdbuzzfeed



Watch Ed and Jeff with Charles Sabine present news, interviews and features in Oz Buzz on YouTube

8:57 - Once we have drugs, how do we know how much to give people? Karl Kiebert suggests some techniques.

9:05 - We'll be interviewing Robert Pacifici, Chief Scientific Officer of CHDI live on stage later. Send your questions about drugs & trials!

9:12 - 'There is nothing more precious to a drug hunter than an observation made in the population you want to treat' Robert Pacifici, CHDI

9:15 - So sign up for [observational](#) trials! PREDICT-HD and TRACK-HD are two options

9:31 - Another [observational](#) trial to consider - ENROLL-HD

9:33 - 'There's no such thing as a good or bad animal model of HD' - they all tell us useful things about different aspects - Pacifici

9:34 - We must understand how our experimental drugs work, and what problems they're targeting, if we're to test them successfully - Pacifici

9:46 - Joaquim Ferreira - It's time to face challenge of how to do drug trials in ppl with the Huntington's disease mutation but no symptoms

9:48 - Ferreira - designing trials carefully can help us tell the difference between effects on symptoms, and altering the progression of HD

9:52 - Studies like PREDICT, TRACK and ENROLL are crucial for getting to trials in pre-onset HD, & getting enough participants - Ferreira

9:59 - Drug regulatory agencies are willing to consider new rules for testing drugs in HD before symptom onset - if the community is on board

10:04 - "There are an impressive number of things moving towards trials that were specifically designed with Huntington's in mind" - Pacifici

10:47 - **Jeff:** Steve Finkbeiner has built robot microscopes to understand how mutant huntingtin kills cells. Really cool.

10:51 - Jeff is now reporting from the session on 'Basic science: protein homeostasis'. Ed reporting on 'Biomarkers'

10:53 - **Ed:** Functional [MRI](#) scanning reveals important brain changes in pre-onset HD. Could be important for PreHD trials- Dr Nellie Georgiou

11:02 - **Jeff:** Question your assumptions. Steve Finkbeiner says that things we once thought bad for [neurons](#) might actually be protecting them

11:07 - **Ed:** Brain scans have helped develop drugs in diseases like Parkinson's and Alzheimer's. We can learn from that - Dr Rachael Scahill

11:21 - **Ed:** TRACK-HD results show that the earliest brain changes in HD mutation carriers may be in the 'white matter' connections - Scahill

11:23 - **Jeff:** Danny Hatter has built labels that let scientists follow the [huntingtin protein](#) around in live cells

11:25 - **Ed:** More TRACK-HD results: shrinkage in specific brain areas is linked to movement control. Again, may help us test drugs - Scahill

11:28 - **Ed:** "It takes a lot of sleuthing to get measurements precise enough to reveal the effects of drugs" - Prof Julie Stout

11:38 - **Ed:** We're "not too far off" being able to detect drug benefits on thinking skills in HD mutation carriers - Stout

11:40 - **Jeff:** Bev Davidson is working to develop "[RNAi](#)" therapies, which turn off the mutant [huntingtin protein](#)

11:42 - **Jeff:** Bev: Even partial reduction of mutant huntingtin has beneficial effects in HD mice - we don't need to completely 'silence' it.

11:50 - **Ed:** TRACK-HD and 'CAB' project are giving us a toolkit of reliable & meaningful tests for studying cognitive problems in HD - Stout

11:58 - **Jeff:** Bev has been testing '[RNAi](#)' silencing in monkeys, a critical step to setting up human trials. Results

show beneficial effects

“Nobody can handle HD alone - true of at-risk people and care professionals - it takes a great team from early on - Dr Martha Nance ”

12:04 - **Ed:** Huntington’s involves many cell types, not just [neurons](#) - including immune system. A whole-body disease - Prof Paul Muchowski #WHCD

12:07 - **Jeff:** Ralf Reilmann and TRACK-HD have developed machines to measure subtle motor problems in HD, like tongue strength

12:11 - **Ed:** [KMO](#) inhibitor drug, acting on blood immune cells, extends lifespan of HD mice - Muchowski

12:12 - **Jeff:** Reilmann - subtle changes in movements occur early in people carrying the HD mutation, before the onset of full-blown HD

12:14 - **Ed:** Muchowski also working on drugs to target ‘cannabinoid’ receptors (there’s no direct evidence for marijuana benefits in HD though)

12:18 - **Ed:** HD mice genetically engineered to lack ‘CB2’ cannabinoid receptors perform worse on tests of movement function - Muchowski

12:21 - **Jeff:** Reilmann - novel machines to measure HD movement symptoms are already being used in a human HD drug trial in Europe

12:22 - **Ed:** Breaking news. a drug that activates CB2 receptors improves motor function and prolongs the lifespan of HD mice - Muchowski

12:23 - **Ed:** CB2 activator drug even improves symptoms in ‘late stage’ mice - Muchowski

12:24 - **Ed:** Surprisingly the CB2 [receptor](#) isn’t found in the brain - meaning the CB2 drug may be acting in the blood, like the [KMO](#) inhibitor

12:28 - **Ed:** Targeting the immune system directly with an antibody to the immune signaling molecule IL6 produces similar benefits - Muchowski

12:47 - **Ed:** Working in HD fruit flies, Juan Botas found calcium changes. Now using data networks to work out what it means for patients

13:52 - Ed now reporting from the ‘clinical care research’ session. Jeff reporting from ‘Basic science: systems & peripheral pathology’

13:55 - **Jeff:** Maria Bjorkqvist - HD is a whole body disease, not just a brain disease. Patients have problems in bone, fat, muscle & others

13:56 - **Ed:** Regular patient/carer education sessions improve anxiety, mood, coping strategies and quality of life in HD - Prof Raymund Roos

14:00 - **Ed:** Roos - We must not hide from difficult issues like end-of-life care and suicide. Silence is the enemy. Keep talking.

14:03 - **Jeff:** Bjorkqvist - Heart attacks kill a large percentage of HD patients - this could this be part of the disease, not coincidence

14:14 - **Jeff:** Richard Faull - People with HD have diverse symptoms, which causes different patterns of brain cell loss

14:16 - **Jeff:** Faull - Donated human brains from HD patients are a precious gift to scientists studying the disease

14:27 - **Ed:** Dr David Craufurd: We have good treatments for psychiatric problems in HD e.g. depression, anxiety & aggression. Speak to your doc!

14:47 - **Jeff:** George Rebec records the firing of brain cells from awake mice, and can see clear changes in firing patterns in HD mice

14:53 - **Ed:** Problems recognizing other people’s emotions are more widespread than previously thought in HD patients - Izelle Labuschagne

15:17 - **Ed:** Nobody can handle HD alone - true of at-risk people and care professionals - it takes a great team from early on - Dr Martha Nance

15:20 - **Jeff:** William Yang-Building mice with mutant huntingtin in limited brain areas to understand what parts of brain are important in HD

The authors have no conflicts of interest to declare. [For more information about our disclosure policy see our FAQ...](#)



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[World Congress on Huntington's disease 2011 website](#) [Watch Oz Buzz on YouTube](#) [Karl D Kieburtz, MD, MPH, University of Rochester](#) [PREDICT-HD](#) [TRACK-HD](#) [Enroll-HD](#) [Steven Finkbeiner, MD, PhD, Gladstone Institute](#) [Danny Hatters, PhD, Bio21 Institute](#) [Beverly Davidson PhD, Davidson Laboratory](#) [HDBuzz article on Dr Paul Muchowski's KMO work](#)

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- Glossary
- **huntingtin protein** The protein produced by the HD gene.
- **observational** A study in which measurements are made in human volunteers but no experimental drug or treatment is given
- **Receptor** a molecule on the surface of a cell that signalling chemicals attach to
- **neuron** Brain cells that store and transmit information
- **RNA interference** A type of gene silencing treatment in which specially designed RNA molecules are used to switch off a gene
- **magnetic resonance** A technique using powerful magnetic fields to produce detailed images of the brain in living humans and animals
- **KMO** kynurenine mono-oxygenase, an enzyme that controls the balance of harmful and protective chemicals resulting from the breakdown of proteins

- [Read more definitions in the glossary](#)

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Generated on April 17, 2019 — Downloaded from <https://en.hdbuzz.net/047>