Oz Buzz Updates: Day 2

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

By Professor 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 Kieburtz 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...

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

---