HD Therapeutics Conference 2012 Updates: Day 1

Day 1 of our coverage of the Huntington's Disease Therapeutics Conference

By Dr Jeff Carroll | **February 29, 2012** | **Edited by Professor Ed Wild**

Our first daily report from the annual Huntington's Disease Therapeutics Conference in Palm Springs, California. We'll be bringing you live updates via Twitter over the next two days. You can tweet @HDBuzzFeed or email palmsprings@hdbuzz.net with your questions, comments and queries.

Monday, February 27, 2012

17:02 - The Huntington's Disease Therapeutics Conference has officially begun. Ed and Jeff will be tweeting every day

The Huntington's Disease Therapeutics Conference, in Palm Springs, California, is a gathering of the world's top HD researchers

Image credit: Gene Veritas

17:06 - Dr Robert Pacifici opens the conference. Reviews the many 'impossible' questions science has already answered in HD

17:00 - CHDI Chief Scientific Officer Robert Pacifici asks - what are the remaining provocative questions in Huntington's Disease research?

Tuesday, February 28, 2012

9:05 - Good morning from Palm Springs. The science sessions of the HD Therapeutics conference are about to begin.

9:20 - The first session is systems biology - using powerful technology to collect and analyse huge amounts of data to help understand HD

9:25 - **Leroy Hood** (Institute for Systems Biology): scientists should work with mathematicians, engineers, physicists for best chance of progress

9:40 - **Hood**: we should be able to develop chemical 'fingerprints' in blood to diagnose and monitor brain disease.

9:47 - **Hood**: we can now sequence people's entire genomes to look for genes that might affect when and how Huntington's affects people

10:05 - **Keith Elliston** is CHDI's new Vice President of 'Systems Biology' - or the science of understanding entire biological systems.

10:00 - **Elliston**: All the changes we see in HD patients are due to a single mutation, which should help us understand the disease.

10:20 - **Elliston**: CHDI's strategy for developing HD treatments will extensively use systems biology and the deep understanding it can bring

10:22 - CHDI is the non-profit biotech company behind the conference and biggest HD research organisation. We're interviewing their top scientists later.

10:24 - **Elliston**: there is no such thing as a perfect disease model. We need to take the best from each model - and remember patients

10:39 - CHDI is developing a 'map' of all the changes in HD, starting with the function of synapses - chemical connections between neurons

10:43 - **Elliston**: Working to ensure that HD researchers have access to as much information as possible, rather than keeping things private

11:30 - **Jim Gusella** (Massachusetts General Hospital in Boston): the smaller of a person's two CAG repeat lengths does not affect disease onset. See our article on this "New analysis suggests 'small' CAG length doesn't matter after all"

11:58 - **Gusella**: His team is looking at the whole genome of HD patients to try and find other genes that might change HD symptoms.

> **"RNA drugs currently being tested in cystic fibrosis/Duchenne muscular dystrophy might prove useful for HD "**

12:26 - **Gusella**: some of the genes described to change the age that people experience HD symptoms might be wrong, new studies are on going

12:33 - **Hanchuan Peng** (Howard Hughes Medical Institute) studies the shape of brains at the level of individual cells. Could this help HD research?

14:31 - **Melissa Moore** (University of Massachusetts Medical School): The RNA 'message molecule' that tells cells to make Huntingtin protein might cause problems in its own right

14:33 - **Moore**: cells have quality control mechanisms for RNA message molecules. Could we enhance these to reduce damage in HD?

14:51 - **Moore**: RNA drugs currently being tested in cystic fibrosis/Duchenne muscular dystrophy might prove useful for HD

15:01 - **Moore**: lots of possible ways that drugs might be able to reduce production of Huntingtin protein. Gene silencing by the back door?

15:22 - **Naoko Tanese** (New York University School of Medicine): New role for the Huntingtin protein. It acts like a bus, carrying RNA molecules around the cell

15:36 - **Tanese**: one of the RNA molecules that the protein carries around the cell is the Huntingtin RNA. Huntingtin drives its own bus!

16:07 - **Lisa Ellerby** (The Buck Institute for Age Research) studies chemical modification of the Huntingtin protein - tiny tags that change its location and function

16:17 - **Ellerby**: Putting more 'phospho' groups on the Huntingtin protein might make it less toxic. But are there drugs that can do this?

16:37 - **Dimitri Krainc** (Massachusetts General Hospital): studies modification of the Huntingtin protein that direct it to the cells garbage can, clearing it from cells

16:53 - **Krainc**: Drugs developed by CHDI to increase traffic of the mutant Huntingtin protein to the trash work in cells

17:19 - **Marcy MacDonald** (Massachusetts General Hospital) and her group are making buckets of purified Huntingtin protein to study - tough work bit very useful for research

17:36 - **MacDonald**: Adding 'phospho' groups to the Huntingtin protein might be good or bad, we need more information to decide what to target

Sunset conclusions

The morning session reminded us that we must remember that virtually every cell and molecule in our bodies is connected to every other in some way. Focusing exclusively on changing one thing without considering knock-on effects of those changes could lead to unpredictable results. In the afternoon, we heard some intriguing ideas of how cells produce and 'tag' the mutant huntingtin protein - the molecule at the heart of the network of damage in HD - and how we might be able to fine tune those changes to our advantage.

Dr Wild and Dr Carroll's registration fee for the Therapeutics Conference has kindly been waived by CHDI Foundation, Inc., sponsors of the Conference, but their attendance is supported by HDBuzz and the European HD Network, from funds independent of CHDI. CHDI has no input into the selection of subjects or the content of coverage on HDBuzz. For more information about our disclosure policy see our FAQ...

GLOSSARY

huntingtin protein The protein produced by the HD gene.

gene silencing An approach to treating HD that uses targeted molecules to tell cells not to produce the harmful huntingtin protein

therapeutics treatments

CAG repeat The stretch of DNA at the beginning of the HD gene, which contains the sequence CAG repeated many times, and is abnormally long in people who will develop HD

neuron Brain cells that store and transmit information

genome the name given to all the genes that contain the complete instructions for making a person or other organism

RNA the chemical, similar to DNA, that makes up the 'message' molecules that cells use as working copies of genes, when manufacturing proteins.

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