

## EHDN2021 Remote Meeting, Sep. 9 – 11

Meeting Host: Patrick Weydt

### THURSDAY, SEPTEMBER 9th, 2021

**14:00 - 14:30 WELCOME AND INTRODUCTION + EHDN Business (Constitution + Voting Results)**

Anne Rosser, Astri Arnesen, Patrick Weydt

**14:30 - 15:00 KEYNOTE SESSION I: From fruitfly biorhythm genetics to Huntington's**

Charalambos P. Kyriacou

**15:00 - 16:00 PLENARY SESSION I: Genetic modifiers of HD: DNA damage and somatic expansion of repeats**

Chaired by Lesley Jones and Davina Hensman

**1. Gene editing for expanded CAG/CTG repeat disorders: making a long story short (20 min)**

Vincent Dion

**2. Using histone deacetylase 3 inhibition to rein in HD expansion mutations (20 min)**

Bob Lahue

**3. Dissecting Genetic Modifiers of HD: Towards Understanding Mechanism (20 min)**

Vanessa Wheeler

**16:00 - 16:30 BREAK**

**16:30 - 16:35 Announcement: HDYO donation**

Matthew Ellison

**16:35 - 17:35 PLENARY SESSION II: Cognition**

Chaired by Jaime Kulisevsky and Saul Martinez-Horta

**1. Session presentation (5 min)**

Saul Martinez-Horta

**2. Visual system integrity in Huntington's disease (20 min)**

Michael Orth

**3. Hippocampal-dependent memory in Huntington's disease (20 min)**

Julie Stout

**4. Q&A (10 min)**

**5. Closing remarks (5 min)**

Jaime Kulisevsky

**17:35 - 18:35 SHORT COMMUNICATIONS I**

Chaired by: Esther Cubo and Lauren Byrne

**1. Research Participation: The View of Persons at Risk and Persons with Premanifest HD**

Filipa Júlio

**2. Huntington's Disease Burden of Illness (HDBOI): Study Methodology, Sample Representativeness and Fieldwork Risk Mitigation Strategy During the COVID-19 Pandemic**

Idaira Rodríguez

**3. Safety and Tolerability of Lumbar Punctures (LP) Procedure in Patients with HD**

Yara Hassan

**4. Skill-Based Dysphagia Training as an Intervention for Individuals with HD**

Emma Burnip

**5. Introducing JOIN-HD: The Juvenile Onset Initiative for Huntington's Disease**

Rebecca Mason

**18:35 - 18:50** **ROUND-UP and Goodbye for the day**  
Host Patrick Weydt and guest(s) of the day

**18:50-19:00** **BREAK**

**19:00 - 20:00** **POSTER SESSION: meet the poster presenter**

**FRIDAY, SEPTEMBER 10th, 2021**

**13:00-14:00** **POSTER SESSION: meet the poster presenter**

**14:00 - 14:05** **Announcement: MDS/EHDN teaching course**  
Michael Orth

**14:05 - 15:35** **PLENARY SESSION III: Clinical Trial Session**  
Chaired by: Ralf Reilmann and Dina de Sousa

**1. Roche – An update from the tominersen clinical development programme (15 min)**

Scott Schobel

**2. SIGNAL/Vaccinex – results and lessons learnt (15 min)**

Maurice Zauderer

**3. SELECT-HD, an adaptive first-in-human clinical trial to evaluate WVE-003, an investigational allele-selective mHTT-lowering oligonucleotide, in early manifest Huntington's disease (15 min)**

Vissia Viglietta

**4. UniQure – An update on HD-GenetrX-1 and HD-GenetrX-2 - Phase 1 / 2 Clinical Trials of AMT-130 Gene Therapy for early stage HD (15 min)**

David Cooper

**5. Development of TTX-3360 to prevent onset and/or progression in HD and other Repeat Expansion Disorders by Halting Somatic Expansion (15 min)**

Irina Antonijevic

**6. PROOF-HD – update on the status of the phase III clinical trial of pridopidine aiming to improve global function in early stage HD (15 min)**

Michael Hayden

**15:35 - 15:45** **BREAK**

**15:45 - 16:30** **PANEL DISCUSSION: What are the lessons learned in the drug development process?**

Chaired by: Ralf Reilmann and Dina de Sousa

With the Speakers from Plenary Session III

**16:30 - 16:45** **BREAK**

**16:45 - 17:45** **PARALLEL SESSIONS**

**Neuro-Development**

Chaired by: Sandrine Humbert and Silvia Gines

**1. Huntington's disease alters cortical development in mouse and human (20 min)**

Monia Barnat

**2. Pediatric onset Huntington disease: neuro developmental delay or early**

**Multidisciplinary care**

Chaired by: Marleen van Walsem and Ruth Veenhuizen

**1. Introduction (5 min):** Multidisciplinary care and treatment: focus on care in advanced stages: challenges and approaches for managing  
**2. Challenges and practise-based approaches in professional advanced care (15 min)**

Marleen van Walsem

neurodegeneration? (20 min)

Ferdinando Squitieri

**3. Specific developmental alterations of striatal subpopulations in Huntington's disease (20 min)**

Josep Canals

**3. Patients with Huntington's disease living in a nursing home: characteristics, functioning, and gender differences (15 min)**

Ruth Veenhuizen

**4. Passivities of daily living (15 min)**

Manon van Kampen

**5. Discussion (10 min)**

**17:45 - 18:45 SHORT COMMUNICATIONS II**

Chaired by: Ahmad Aziz and Maria Björkqvist

**1. Fronto-Striatal Circuits for Cognitive Flexibility in far from Onset Huntington's Disease: Evidence from the Young Adult Study**

Christelle Langley

**2. Mutation-Related Apparent Myelin, not Axon Density, Drives White Matter Pathology in Premanifest Huntington's Disease: Evidence from in Vivo Ultra-Strong Gradient MRI**

Chiara Casella

**3. A Different Depression: Antidepressant Efficacy and Cognitive Mechanisms of Mood Disorder in Huntington's Disease**

Duncan McLauchlan

**4. Clinical Translation of Stem Cell Therapies for Huntington's Disease (HD)**

Anne Rosser

**5. Triheptanoin is Associated with Clinical Stability and Decreased Caudate Atrophy in HD**

Fanny Mochel

**18:45 - 19:00 ROUND-UP and Goodbye for the day**

Host Patrick Weydt and guest(s) of the day

**19:00 - 20:00 POSTER SESSION: meet the poster presenter**

**SATURDAY, SEPTEMBER 11th, 2021**

**13:00 - 14:00 POSTER SESSION: meet the poster presenter**

**14:00 - 15:00 SHORT COMMUNICATIONS III**

Chaired by: Kathrin Reetz and Nayana Lahiri

**1. FAN1 Controls CAG Repeat Expansion in Huntington's Disease by Dual Functions, MLH1 Retention and Nuclease Activity**

Joseph Hamilton

**2. Striatal Procedural Memory-Induced Transcriptome and Epigenome are Severely Impaired in Huntington's Disease Mice**

Rafael Alcalá-Vida

**3. A New In Vivo and In Vitro Single-Cell Atlas of Developing Medium Spiny Neurons to Guide Future Improvements for Huntington Disease Cell-Replacement Therapies and Disease Modelling**

Vittoria Bocchi

**4. Huntingtin-Mediated Axonal Transport Requires Arginine Methylation by PRMT6**

Alice Migazzi

**5. The 'Hold me Tight' Relationship Program for Couples Facing Huntington's Disease**

Lucienne van der Meer

**15:00 - 16:00 PARALLEL SESSIONS**

**Digital Technologies to Advance Assessment and Care in HD**

Chaired by Alzbeta Mühlbäck and Monica Busse

**1. Introduction to the session: Landscape View of the Field (15 min)**

Monica Busse

**2. IDEA-FAST (15 min)**

Ralf Reilmann

**3. eHealth Platform for HD overview (10 min)**

Pearl van Lonkhuizen

**4. DOMINO-HD overview (10 min)**

Philippa Jones

**5. Panel Discussion: Ethical & practical challenges (10min)**

All speakers and chairs

**Sleep, circadian rhythm and metabolism in HD**

Chaired by: Patrick Weydt and Asa Petersen

**1. Introduction to the topic by the chairs**

**2. Sleep and metabolism in Huntington disease (15 min)**

Roger Barker

**3. Clock genes and circadian rhythm (15 min)**

Charalambos P. Kyriacou

**4. Targeting cholesterol metabolism as a therapy for Huntington disease (15 min)**

Sandrine Betuing

**5. Summary and practical tips (15 min)**

**16:00 - 16:15 BREAK**

**16:15 - 17:45 PLENARY SESSION IV: Upcoming Clinical Trials**

Chaired by: Jean-Marc Burgunder and Sarah Tabrizi

**1. SOM Biotech (15 min)**

TBD

**2. Novartis (15 min)**

Beth Borowsky

**3. Voyager (15 min)**

Maria Lopez-Bresnahan

**4. PTC (15 min)**

Brian Pfister

**5. Enroll-HD Platform Studies (10 min)**

Olivia Handley

**6. HDClarity (5 min)**

Ed Wild

**7. Panel discussion (15 min)**

all speakers from Plenary Session IV

**17:45 - 18:00 BREAK**

**18:00 - 18:45 KEYNOTE SESSION II: Outlook**

Chaired by: Dina de Sousa and Giorgos Papantoniou

**New staging system in HD extending the window of testing and treatment**

Cristina Sampaio (30 min)

Q&A (15 min)

**18:45 - 19:15 ROUND-UP and Goodbye for the day**

**Historical perspective (10 min)**

Tom Bird

**Goodbye (5 min)**

Host Patrick Weydt with Astri Arnesen and Matthew Ellison