

## 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 fruit fly 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**  
Vincent Dion
  - 2. Using histone deacetylase 3 inhibition to rein in HD expansion mutations**  
Bob Lahue
  - 3. Dissecting Genetic Modifiers of HD: Towards Understanding Mechanism**  
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 Jamie Kulisevsky and Saul Martinez-Horta
- 1. Session presentation**  
Saul Martinez-Horta
  - 2. Visual system integrity in Huntington's disease**  
Michael Orth
  - 3. Hippocampal-dependent memory in Huntington's disease**  
Julie Stout
  - 4. Q&A**
  - 5. Closing remarks**  
Jaime Kulisevsky
- 17:35 - 18:35 SHORT COMMUNICATIONS I**  
Chaired by: Esther Cubo and Lauren Byrne
- 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 study update**

Scott Schobel

**2. SIGNAL/Vaccinex – results and lessons learnt**

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**

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**

David Cooper

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

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**

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**

Monia Barnat

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

Ferdinando Squitieri

**3. Specific developmental alterations of striatal subpopulations in Huntington's disease**

Josep Canals

**Multidisciplinary care**

Chaired by: Marleen van Walsem and Ruth Veenhuizen

**1. Introduction:** Multidisciplinary care and treatment: focus on care in advanced stages: challenges and approaches for managing

**2. Challenges and practice-based approaches in professional advanced care**

Marleen van Walsem

**3. Neuro-affective approach in day-to-day care**

Heidi Møller-Lund

**4. Passivities of daily living**

Manon van Kampen

**5. Discussion**

- 17:45 - 18:45 SHORT COMMUNICATIONS II**  
Chaired by: Ahmad Aziz and Maria Björkqvist
- 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: Hoa Nguyen and Kathrin Reetz

- 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**

Monica Busse

**2. IDEA-FAST**

Ralf Reilmann

**3. eHealth Platform for HD overview**

Pearl van Lonkhuizen

**4. DOMINO-HD overview**

Philippa Jones

**5. Panel Discussion: Ethical & practical challenges**

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**

Roger Barker

**3. Clock genes and circadian rhythm**

Charalambos P. Kyriacou

**4. Targeting cholesterol metabolism as a therapy for Huntington disease**

Sandrine Betuing

**5. Summary and practical tips**

- 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**

TBD

**2. Novartis**

Beth Borowsky

**3. Voyager**

Maria Lopez-Bresnahan

**4. PTC**

Brian Pfister

**5. Enroll-HD Platform Studies**

Olivia Handley

**6. HDClarity**

Ed Wild

**7. Panel discussion**

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**

Swati Sathe

**Q&A**

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

**Historical perspective**

Tom Bird

**Goodbye**

Host Patrick Weydt with Astri Arnesen and Matthew Ellison