

EHDN Plenary Meeting, Sep. 16 – 18, 2022, Bologna, Italy

FRIDAY, SEPTEMBER 16th, 2022

07:30 – 08:30 **REGISTRATION DESK**

08:30 – 09:00 **WELCOME AND INTRODUCTION**

Chaired by Anne Rosser, Patrick Weydt, Astri Arnesen

09:00 – 10:30 **PLENARY SESSION I: 150 years of Huntington disease**

Chaired by Anne Rosser and Charles Sabine

1. Living with HD

Charlotte Raven and Ed Wild (30 min)

2. Mutant Huntingtin: Molecules, Models, Medicines

Gillian Bates (30 min)

3. Wild-type Huntingtin: Evolution, Structure, Function

Elena Cattaneo (30 min)

10:30 – 11:00 **BREAK**

11:00 – 12:30 **PLENARY SESSION II: New genetics of HD**

Chaired by Hoa Huu Phuc Nguyen and Flaviano Giorgini

1. Genetic Modifiers in HD and somatic mosaicism

Darren Monckton (30 min)

2. Non-pathogenic CAG tract and gray matter microstructure in human brain

Larissa Arning (20 min)

3. FAN1 as genetic modifier for HD progression

Joe Hamilton (20 min)

4. Genomic modifiers in clinical practice and therapy

Galen Wright (20 min)

12:30 – 14:00 **LUNCH and POSTER VIEWING**

14:00 – 15:00 **PARALLEL SESSIONS I**

A. HTT structure and function

Chaired by Chiara Zuccato and Frederic Saudou

1. HTT/HAP40 complex in the pathophysiology of HD

Stefan Kochanek (20 min)

2. HTT role in neuronal transport

Frederic Saudou (20 min)

3. HTT exon 1 aggregation and PTMs code

Hilal Lashuel (20 min)

B. Digital Endpoints

Chaired by Ralf Reilmann and Ahmad Aziz

1. Digital endpoints – a perspective from Multiple Sclerosis bridging to HD

Marcus D'Souza (20 min)

2. Lessons learnt from the Roche HD digital monitoring programme

Jonas Dorn (20 min)

3. Lessons learnt from Q-Motor and Q-Cog in HD trials

Robin Schubert (20 min)

15:00 – 15:30 **BREAK**

15:30 – 17:00 **PLENARY SESSION III: Approaches to quality of life and clinical management of HD**

Chaired by Alzbeta Mühlbäck and Giorgos Papantoniou

1. Session Introduction

Alzbeta Mühlbäck (5 min)

2. Coping with Symptoms of HD – Patient Story

Tess Persson (10 min)

3. Quality of Life and PROMs for HD

Steve McKenna (20 min)

4. Understanding Emotions and Psychiatric Aspects of HD

Åsa Petersen (20 Min)

5. Management of the Psychiatric Symptoms in HD

Duncan McLauchlan (20 min)

6. Panel Discussion (15 min)

17:00 – 17:30 **BREAK**

17:30 – 18.30 **Working Groups Session**

Chaired by Michael Orth and Kristina Becanovic

18:30 – 19:15 **SHORT COMMUNICATIONS I**

Chaired by Maria Björkqvist and Marco Onorati

19:15 – 20:00 **Evening session: Special Lecture by Nora Guthrie**

Chaired by Michaela Winkelmann and Patrick Weydt

20:00 **DINNER ON YOUR OWN**
Gene banner and lay event

SATURDAY, SEPTEMBER 17th, 2022

08:00 – 09:15 **MEET THE EXPERT BREAKFAST SESSION***

09:30 – 11:00 **PLENARY SESSION IV: Update on ongoing clinical trials**

Chaired by Sarah Tabrizi and Astri Arnesen

1. Prilenia

Michael Hayden (10 min)

2. UniQure

David Cooper (10 min)

3. Wave

Michael Panzara (10 min)

4. PTC: An Update to PIVOT (Phase 2 study of PTC518 in Huntington's Disease)

Amy-Lee Bredlau (10 min)

5. Novartis

Beth Borowsky (10 min)

6. SOM Biotech

Catherine Scart (10 min)

7. Panel Discussion (30 min)

11:00 – 11:30 **BREAK**

11:30 – 12:15 **SHORT COMMUNICATIONS II**

Chaired by Katrin Lindenberg and Anne Norremolle

12.15 – 13.00 **Business Meeting**

Chaired by Anne Rosser

13.00 – 14:30

LUNCH and POSTER VIEWING

14:30 – 15:30

PARALLEL SESSION II

A. The use of biomarkers in clinical trials of neurodegenerative disorders

Chaired by Maciej Figiel and Lauren Byrne

1. Neurofilaments as biomarkers across neurodegenerative disorders

Henrik Zetterberg (30 min)

2. A novel ultrasensitive huntingtin assay to measure huntingtin protein in a polyglutamine length-independent manner

Douglas Macdonald (15 min)

3. An update on iMAGeMHTT - mHTT PET tracer development in the clinic

Aline Delva (10 min)

4. Outro - a perspective of unmet biomarker needs for HD therapeutic development

Lauren Byrne (5 min)

B. Neurodevelopment/Pediatric/juvenile HD

Chaired by Sandrine Humbert and Peggy Nopoulos

1. Axonal growth defects during development contribute to early HD signs

Mariacristina Capizzi (20 min)

2. Growth and development of the striatum in children and adolescents at risk for HD

Peggy Nopoulos (20 min)

3. Huntington disease in Kids

Ferdinando Squitieri (20 min)

15.30 – 15:45

BREAK

15:45 – 16:45

PARALLEL SESSION: III

A. White matter changes in HD

Chaired by Åsa Petersen and Mahmoud Pouladi

1. Oligodendrocyte dysfunction in Huntington disease

Mahmoud Pouladi (20 min)

2. Glial-specific transcriptomic changes in HD

Anna Williams and Sunniva Bøstrand (20 min)

3. Imaging white matter changes in HD

TBD (20 min)

B. Multidisciplinary care

Chaired by Ruth Veenhuizen and Monica Busse

1. Session Introduction (5 min)

2. Moral deliberation and dilemmas in multidisciplinary care

Ruth Veenhuizen (10 min)

3. Moving against resistance: implications for physiotherapy and physical activity

Una Jones (12 min)

4. Managing the transition from independence

Manon van Kampen (12 min)

5. Nutrition decision making

Margret Knoll (12 min)

6. Panel Discussion (10 min)

17:00 – 17:45

PLENARY LECTURE I: Lessons learnt from other neurodegenerative disorders: Protein vaccination to treat RAN translation in C9ORF ALS

Chaired by Patrick Weydt

Speaker: Dieter Edbauer

17:45 – 18:15

PLENARY LECTURE II: HD-ISS - a primer for use in clinical research

Chaired by Cristina Sampaio

Speaker: Sarah Tabrizi

18:15 – 19:00

ROUND TABLE DISCUSSION: What do results from clinical trials mean?

Chaired by Jean-Marc Burgunder

Speaker: Serge Bakchine, Henrik Zetterberg, Bernhard Landwehrmeyer and Jessica Koehli

19:00 – 23:00

NETWORKING EVENING

Finger Food, Drinks & Music // Photo exhibition 'Abandonment: From Landscapes to Mindscapes' by EHA

SUNDAY, SEPTEMBER 18th, 2022

08:00 – 09:15 MEET THE EXPERT BREAKFAST SESSION*

09:30 – 11:00 PLENARY SESSION V: Advanced Therapies for Huntington's Disease

Chaired by Anne Rosser and Romina Aron-Badin

1. **Introduction – overview of challenges in delivering advanced therapies to the brain in HD**
Romina Aron-Badin (15 min)
2. **How do we innovate? Challenges of delivering substances to the brain and trial design issues**
Liam Gray (15 min of delivery)
Cheney Drew (15 min on trial design)
3. **What is new? Example of cell therapy**
Leslie Thompson (15 min)
4. **What is new? DBS**
Jan Vesper (15 min)
5. **Discussion** (15 min)

11:00 – 11:30 BREAK

11:30 – 12:30 PLENARY SESSION VI: Update on planned clinical trials

Chaired by Tim McLean and Bernhard Landwehrmeyer

1. **AskBio**
Nathalie Cartier-Lacave (13 min)
2. **Sage**
Aaron Koenig (13 min)
3. **Roche: A Phase II dose-finding study to explore the safety, biomarkers and efficacy of tominersen in participants with early manifest and prodromal Huntington's disease**
Peter McColgan (13 min)
4. **Annexon**
Henk-André Kroon (13 min)

12:30 – 13:00 LATE BREAKING NEWS: TBD

Chaired by TBD

13:00 – 13:30 ROUND-UP AND POSTER AWARDS

Chaired by Patrick Weydt and Åsa Petersen

*MEET THE EXPERT BREAKFAST SESSIONS:

1. **HD in Italy and in Italian**, chaired by Chiara Zuccato and Antonio Fontana➔ **Saturday, Sept 17th, 8:00 – 9:15: Integrated Care Pathway for Huntington's Disease //
Percorso Diagnostico Terapeutico Assistenziale per la Malattia di Huntington**

Guest: Cesa Scaglione

➔ **Sunday, Sept 18th, 8:00 - 9:15: Basic Research and Clinical Trial //
Ricerca di Base e Sperimentazioni Cliniche**

Guest: Caterina Mariotti

2. **HD for beginners**, chaired by Ahmad Aziz and Bernhard Landwehrmeyer3. **Young people and HD**, chaired by Jenna Heilman and Rebecca Mason from HDYO4. **Psychiatric Session about sexuality and intimacy**, chaired by Alzbeta Mühlbäck and Rainer Hoffmann5. **Eating and Swallowing**, chaired by Falk Schradt and Angela Nuzzi