

WEDNESDAY, AUGUST 24

4:00 - 10:00 PM REGISTRATION (Grand Ballroom Foyer)

WEDNESDAY, AUGUST 24 - EVENING SESSION (Grand Ballroom)

6:00 - 8:00 PM: Kickoff Session - Orientation About Huntington's Disease
(Chair: Leslie Thompson)

6:00 - 6:10 PM: Meghan Donaldson and Leslie Thompson: WELCOME

6:10 - 6:55 PM: Sarah Tabrizi: *Identifying the ideal time to treat Huntington's disease to prevent neurodegeneration - where are we now?*

6:55 - 7:40 PM: Peter McColgan: *Towards an understanding of the treatment and mechanistic effects of tominersen*

7:40 - 8:10 PM: Discussion

8:10 - 10:00 PM RECEPTION (Grand Ballroom Foyer)

THURSDAY, AUGUST 25

8:00 - 9:00 AM BREAKFAST (Grand Ballroom Foyer)

THURSDAY, AUGUST 25 - MORNING SESSION (Grand Ballroom)

9:00 - 10:15 AM: Welcome

9:00 - 9:10 AM: Anne Young: Welcome and Introduction

9:10 - 10:15 AM: Living with Huntington's Disease: Family Perspectives
Diana Rosas in conversation with individual living with HD

10:15 - 10:45 AM BREAK (Grand Ballroom Foyer)

10:45 AM - 12:30 PM: Therapeutics - Clinical Trials, Part I: *Treating early (phenotyping and endpoints)*

(Chairs: Beverly Davidson, Diana Rosas, Sarah Tabrizi)

10:45 - 11:05 AM: Jeff Long: *Clinical trial planning with the Huntington's Disease Integrated Staging System*

11:05 - 11:25 AM: Steve Finkbeiner: *Artificial intelligence to detect and quantify phenotypes in disease models and patients*

11:25 - 11:45 AM: Ed Wild: *HD biomarkers: a midterm report card*

11:45 - 12:00 PM: Special Presentation

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12:00 - 12:30 PM: Discussion

12:30 - 1:30 PM: BOXED LUNCH (Riverview & Parkview – East Tower, Level II)

POSTER SET-UP (Abstracts #1-64) (Skyline Suites)

THURSDAY, AUGUST 25 – AFTERNOON SESSION (Grand Ballroom)

1:30 - 3:10 PM: Therapeutics - Clinical Trials, Part II

(Chairs: Beverly Davidson, Diana Rosas, Sarah Tabrizi)

1:30 - 2:20 PM: Clinical Trials – Active, Session 1

1:30 - 1:40 PM: Irina Antonijevic, Triplet: SHIELD HD – A natural history study to support clinical development of TTX-3360 in HD and other repeat expansion disorders

1:40 - 1:50 PM: Ricardo Dolmetsch, UniQure: HD-GeneTRX-1 and HD-GeneTRX-2: Phase 1/2 clinical trials of AMT-130 gene therapy for early-stage HD

1:50 - 2:00 PM: Amy-Lee Bredlau, PTC: PIVOT HD trial: Updates on PIVOT, PTC518-CNS-002-HD

2:00 - 2:20 PM: Discussion

2:20 - 3:20 PM: Clinical Trials – Active, Session 2

2:20 - 2:30 PM: Danlin Xu, Wave Life Sciences: SNP-targeting with WVE-003 as an investigational therapeutic approach to selective mHTT-lowering in Huntington's disease

2:30 - 2:40 PM: Ellen Cahir-MacFarland, Annexon: Stop at the start: The therapeutic potential of C1q inhibition in Huntington's disease

2:40 - 2:50 PM: Aaron Koenig, Sage: PERSPECTIVE PROGRAM: Evaluating SAGE-718, A novel, investigational NMDAR PAM, in Huntington's disease cognitive impairment

2:50 - 3:00 PM: Michael Hayden, Prilenia: The PROOF phase 3 study in HD: Looking back, looking forward

3:00 - 3:20 PM: Discussion

3:20 - 3:50 PM BREAK (Grand Ballroom Foyer)

3:50 - 6:30 PM: Systems Biology Approaches to Study HD; Glial Cell Biology in HD

(Chairs: Steve Finkbeiner, William Yang)

3:50 - 4:35 PM: Steve McCarroll, Keynote: Insights from single-cell analysis of CAG repeat expansion and genome-wide RNA expression

4:35 - 4:55 PM: Myriam Heiman: Single-cell dissection of the human brain vasculature in health and HD

4:55 - 5:15 PM: Karine Merienne: Epigenetic gene reprogramming in Huntington's disease

5:15 - 5:35 PM: Michelle Gray: Astrocyte-neuron interactions in Huntington's disease

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5:35 - 5:55 PM: Baljit Khakh: *Dissecting neuronal and astrocytic contributions to HD pathophysiology in mice with zinc finger protein transcriptional repressors*

6:00 - 6:30 PM: Discussion

6:30 - 10:00 PM DINNER AND POSTER VIEWING (Abstracts #1-64) (Skyline Suites)

FRIDAY, AUGUST 26

8:00 - 9:00 AM BREAKFAST (Grand Ballroom Foyer)

TAKE DOWN POSTERS (Abstracts #1-64)

FRIDAY, AUGUST 26 – MORNING SESSION (Grand Ballroom)

9:00 - 10:35 AM: Pre-Clinical Programs for Clinical Development

(Chairs: Beverly Davidson, Diana Rosas, Sarah Tabrizi)

9:00 - 9:20 AM: Anastasia Khvorova: *RNAi-based modulation of Huntingtin mRNA variant expression and somatic repeat expansion: the future for disease-modifying therapeutics*

9:20 - 9:40 AM: Kai Wang: *Haplotyping SNPs for allele-specific gene editing of the mutant Huntingtin allele using long-read sequencing*

9:40 - 10:00 AM: Alex Mas Monteys: *Regulated editing of mutant Huntingtin using Xon*

10:00 - 10:20 AM: Anne Rosser, Stem Cells 4 HD: *Stem cell therapy in Huntington's: prospects and challenges*

10:20 - 10:35 AM: Discussion

10:35 - 11:11 AM: Datablitz Presentations

(Chair: Sarah Hernandez)

10:35 - 10:47 AM: Christian Neri: *Precision systems-modeling and -biology for robust therapeutic advances in Huntington's disease*

10:47 - 10:59 AM: Thai Bao Nguyen: *Huntingtin's interaction with RNA-binding proteins affects RNA-processing and metabolism*

10:59 - 11:11 AM: Veronica Brito: *Post-transcriptional m6A modifications at HTT intron 1: Implications on the generation of pathogenic exon 1 HTT protein*

11:11 - 11:30 AM BREAK (Grand Ballroom Foyer)

11:30 AM - 12:18 PM: Datablitz Presentations, continued

(Chair: Sarah Hernandez)

11:30 - 11:42 AM: David Leib: *Novel AAVs for CNS therapy*

11:42 - 11:54 AM: Sophie St-Cyr: *Temporal phenotypic changes in HD models for preclinical studies*

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11:54 AM - 12:06 PM: Lauren Byrne: *Influencing Huntington's disease monitoring with remote quantification of Neurofilament light protein*

12:06 - 12:18 PM: Amber Southwell: *Robust huntingtin reduction increases stress susceptibility in induced aged Huntington's disease neurons*

12:30 - 1:30 PM BOXED LUNCH (Riverview & Parkview – East Tower, Level II)

POSTER SET-UP (Abstracts #65-121) (Skyline Suites)

FRIDAY, AUGUST 26 – AFTERNOON SESSION (Grand Ballroom)

1:30 - 3:30 PM: Modifiers

(Chairs: Marian DiFiglia, Christopher Pearson)

1:30 - 1:50 PM: William Yang: *Genetic analysis of HD GWAS modifier genes in mouse models*

1:50 - 2:10 PM: Andrew Yoo: *Reversing neurodegeneration in Huntington's disease patient-derived neurons*

2:10 - 2:30 PM: James Gusella: *Huntington's disease: Genetic complexity in a Mendelian disorder*

2:30 - 2:50 PM: Ai Yamamoto: *Modifying HD and other proteinopathies with Alfy*

2:50 - 3:10 PM: Christine Klein: *Elucidating genetic modifiers of age-related penetrance in X-linked dystonia-parkinsonism*

3:10 - 3:30 PM: Discussion

3:30 - 4:00 PM BREAK (Grand Ballroom Foyer)

4:00 - 5:20 PM: Cortical-Basal Ganglia Circuitry - Who is Perpetrator and Who is Victim?

(Chairs: Lynn Raymond, Scott Zeitlin)

4:00 - 4:20 PM: Carlos Cepeda: *Synaptic pathology in HD: Beyond the corticostriatal pathway*

4:20 - 4:40 PM: Lynn Raymond: *Altered cortical sensory processing in mouse models of Huntington's disease*

4:40 - 5:00 PM: Hongwei Dong: *The mouse cortico-basal ganglia-thalamic neural network*

5:00 - 5:20 PM: Discussion

5:20 - 6:00 PM: Datablitz Presentations

(Chair: Sarah Hernandez)

5:20 - 5:32 PM: Ali Khoshnan: *Investigating the toxic properties of mutant Huntingtin in gut-brain pathways*

5:32 - 5:44 PM: Rachel Harding: *Defining a molecular role for the elusive huntingtin protein: insights into huntingtin-nucleic acid interactions*

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5:44 - 5:56 PM: Gong-Her Wu: *CryoET reveals organelle phenotypes in Huntington's disease patient iPSC-derived and mouse primary neurons*

6:30 - 10:00 PM DINNER AND POSTER VIEWING (Abstracts #65-121) (Skyline Suites)

SATURDAY, AUGUST 27

8:00 - 9:00 AM BREAKFAST (Grand Ballroom Foyer)

SATURDAY, AUGUST 27 - MORNING SESSION (Grand Ballroom)

TAKE DOWN POSTERS (Abstracts #65-121)

9:00 - 10:20 AM: Mechanism from Repeat Toxicity Angle from All Diseases
(Chairs: Diane Merry, Rick Morimoto)

9:00 - 9:20 AM: Aaron Gitler: *Expanding mechanisms and therapeutic targets for neurodegenerative disease*

9:20 - 9:40 AM: Laura Ranum: *RAN translation in HD: mechanistic insights and therapeutic opportunities*

9:40 - 10:00 AM: Nancy Bonini: *The impact of m6A epitranscriptomic modification on the Drosophila brain with stress & disease*

10:00 - 10:20 AM: Rick Morimoto: *Polyglutamine proteotoxicity accelerates proteostasis collapse in aging*

10:20 - 10:40 AM BREAK (Grand Ballroom Foyer)

10:40 AM - 12:30 PM: Mechanism from Repeat Toxicity Angle from All Diseases

(Chairs: Diane Merry, Rick Morimoto)

10:40 - 11:00 AM: Janghoo Lim: *Elucidating the neuron-glia interplay underlying spinocerebellar ataxia type 1*

11:00 - 11:20 AM: Hayley McLoughlin: *Impaired oligodendrocyte maturation: an early feature in SCA3 disease pathogenesis*

11:20 - 11:40 AM: Diane Merry: *USP7 - a novel mechanism and potential therapeutic target in SBMA*

11:40 AM - 12:00 PM: Eric Wang: *Supply chain problems: RNA-mediated pathology in repeat expansion disease*

12:00 - 12:30 PM: Discussion for ALL Mechanism Session participants

12:30 - 1:30 PM BOXED LUNCH (Riverview & Parkview – East Tower, Level II)

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SATURDAY, AUGUST 27 - AFTERNOON SESSION (Grand Ballroom)

1:30 - 2:40 PM: Mechanism Session - DNA Repair

(Chairs: Ray Truant, Christopher Pearson)

1:30 - 1:50 PM: Partha Sarkar: *Huntingtin stimulates DNA repair to maintain neuronal genome integrity*

1:50 - 2:10 PM: Vanessa Wheeler: *DNA repair and somatic CAG repeat instability*

2:10 - 2:30 PM: Jeff Carroll: *Continued investigations of the links between huntingtin-lowering treatments and somatic instability in Htt's CAG tract in vivo*

2:30 - 2:42 PM: Amit Deshmukh: *FAN1 nuclease excises excess repeats from slipped-DNAs: A mechanism of repeat instability*

2:42 - 3:10 PM BREAK (Grand Ballroom Foyer)

3:10 - 4:20 PM: Mechanism Session - DNA Repair, continued

(Chairs: Ray Truant, Christopher Pearson)

3:10 - 3:30 PM: Anna Pluciennik: *Crosstalk between DNA repair systems in CAG repeats expansion*

3:30 - 3:50 PM: Christopher Pearson: *Modifiers of repeat diseases are modifiers of repeat expansions*

3:50 - 4:20 PM: Discussion for ALL Mechanism Session participants

4:30 - 5:15 PM: Closing Keynote

Gill Bates: *The incomplete spicing of huntingtin: implications for Huntingtin lowering strategies*

5:15 - 5:45 PM: Special Presentation and Wrap-Up Discussion

6:00 - 7:00 PM RECEPTION (Grand Ballroom Foyer)

7:00 - 10:00 PM SPECIAL LOBSTER DINNER (Grand Ballroom)

With Jang-Ho Cha and Karen Newman, Board Chair