Draft Scientific Program

CONFERENCE: 2nd Conference on Large

Animal Models of Huntington's Disease

LOCATION: Chateau Liblice,

Conference Center of the Czech Academy of Science,

Czech Republic

DATES: November 17-20, 2013

Sunday, November 17

Arrival and Check-in

18:00 pm Welcome drink and dinner

Monday, November 18

7:00 to 9:00 am **Breakfast**

Morning

Welcome / Introductory Comments by Organizers

9:00-9:10 am

• Jan Motlik (Institute of Animal Physiology and Genetics, Czech Republic)

9:10-9:30 am

- David Howland (CHDI Foundation, USA)
 - Modeling in HD: The need for large animals for research and development; a CHDI Perspective

Session I. Neurological Disease, Perspectives from Mouse Models and the Human Condition

Chair: Jenny Morton

9:30-10:00 am

• Jim Gusella (Massachusetts General Hospital, USA)

Perspectives of a Genetic Disease

10:00-10:30 am

• Marcy Macdonald (Massachusetts General Hospital, USA)

Huntington's disease: Genetics-based experimental systems

10:30 – 11:00 am

Coffee Break

11:00-11:30 am

• Petr Vodicka (Massachusetts General Hospital, USA)

Manipulating protein degradation pathways in mouse HD models

11:30-noon

• Marian DiFiglia (Massachusetts General Hospital, USA)

Using synaptosomes to identify changes in HD mouse brain

Noon to 13:30 pm

Lunch

Afternoon

Session II. Emerging Technologies and Development of Large Animal Models of Disease

Chair: Seung Kwak

13:30 pm to 14:00 pm

• John R. Swart (Exemplar Genetics, USA)

Homologous recombination and somatic cell nuclear transfer to generate new knock in HD minipig models

14:00 pm to 14:30 pm

• Paul Verma (South Australia Research and Development Institute and Monash)
Genome editing with TALENs and iPSC to engineer Large Animal Disease Models

14:30 pm-15:00 pm

• Jodi McBride (Oregon National Primate Research Center, USA)
MRI-guided delivery of AAV1-mHTT into the caudate and putamen of rhesus macaques: working towards a new NHP model of Huntington's disease

15:00 pm to 15:30 pm

• Mahmoud A. Pouladi (UBC, Vancouver, Canada and Singapore)
TALENs to make new HD NHP Knock in Models

15:30-16:00 pm

Coffee Break

16:00 pm to 17:00 pm

Discussion Forum

Discussion Leaders: Seung Kwak and Ralf Reilmann

17:00 pm to 19:00 pm

Session III. Poster Viewing with Drinks

19:00 pm to 22:00 pm

Dinner with accompanying alchemistic program and wine tasting

Tuesday, November 19

7:00 to 9:00 am

Breakfast

Morning

Session IV. Translatable Measures: Patients to Large Animal Models and Back Chair: Larry Park (CHDI)

9:00 am to 9:30 am

• Jenny Morton (University of Cambridge, UK)

Behavioral testing in sheep models of neurodegenerative disease

9:30 am to 10:00 am

• Nick Perentos (University of Cambridge, UK)

In vivo electroencephalography and deep brain electrophysiology in sheep models of neurodegeneration

10:00 am to 10:30 am

Coffee Break

10:30 am to 11:00 am

• Ralf Reilmann (University of Muenster, Germany)

TRACK-HD minipig

11:00 am to 11:30 am

• Anthony Chan (Emory University, USA)

HD transgenic NHP for drug discovery

11:30 am to 12:15 pm

Discussion Forum

Discussion Leaders: Larry Park and Marian DiFiglia

12:15 pm to 13:30 pm

Lunch

Session V. Histopathology and Molecular Phenotyping in Large Animal Models

Chair: Jim Gusella

13:30 pm to 13:50 pm

• Hana Hansikova (University of Prague, Czech Republic)

Impaired mitochondrial functions in spermatozoa of TgHD boars

13:50 pm to 14:10 pm

• Stefan Juhas (Institute of Animal Physiology and Genetics, Czech Republic) Immunohistochemistry of F2 generation of minipigs

14:10 pm to 14:30 pm

• Petr Solc (Institute of Animal Physiology and Genetics, Czech Republic)
DNA damage response in Huntington disease

14:30 pm to 15:00 pm

• Susanne Reid (Unversity of Auckland, NZ)

Molecular and histological phenotypes in HD Sheep

15:00 to 15:30 pm

• Russell Snell (Unversity of Auckland, NZ)

Using genetics and complex dataset to discover modifiers and pleiotropic effects

15:30 pm to 16:00 pm

Coffee Break

Session VI. Invasive and Interventional Strategies using Large Animal Models

Chair: Marian DiFiglia

16:00 pm to 16:30 pm

• Matt Gunis (University of Massachusetts, USA)

Finding the striatum using MRI-guided CT in the sheep

16:30 pm to 17:00 pm

• Neil Aronin (University of Massachusetts, USA)

Preclinical testing of AAV-mediated Htt lowering therapies in HD sheep: Trials and Tribulations

17:00 pm to 17:30 pm

• Simon Bawden (South Australia Research and Development Institute, Australia) Challenges and Solutions for using large animal models in therapeutic testing

17:30 pm to 17:45 pm

Coffee Break

17:45 pm to 18:30 pm

Discussion Forum

Discussion leaders: Jenny Morton and Jodi McBride

18:30 pm to 19:00 pm

Closing Remarks: Jan Motlik

19:00 pm to 21:00 pm

Dinner

Wednesday, November 20

7:00 to 9:00 am **Breakfast**

9:00 am to 5:00 pm

- Closed Meeting of the CHDI Large Animal Working Group
 - o Core members invited only

9:00 am to noon

• Open Meeting (Morning only): Young Investigators – Finding Ways to Synergize in Advancing Research for Large Disease Models