

Scientific Program

CONFERENCE: **2nd Large Animal Models
of Neurodegenerative
Diseases**

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
Welcome drink and dinner

Monday, November 18

Morning

Welcome / Introductory Comments by Organizers

- **Jan Motlik, IAPG, CZ**
- **David Howland (CHDI Foundation, Princeton, USA)**
Manipulating protein degradation pathways in mouse HD models. Modeling in HD: The need for large animals for research and development; a CHDI Perspective

Session I. Neurological Disease, Perspectives from Mouse models

Chair: TBD

- **Marcy Macdonald (MGH, Boston)**
Huntington's disease: Genetics based experimental systems
- **Petr Vodicka, Boston, USA, Libechov, Czech Republic**
Manipulating protein degradation pathways in mouse HD models
- **Marian DiFiglia (MGH, Boston)**
Using synaptosomes to identify changes in HD mouse brain

Coffee Break

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

Chair: TBD

- **Andreas Nørgaard Glud, Aarhus, DK**
 - Direct gene transfer in the Gottingen minipig CNS using stereotaxic lentiviral microinjections
- **Mette Slot Nielsen, Aarhus, DK**
 - A Göttingen minipig model of PD based on continuous MPTP intoxication
- **Jodi McBride, Portland, Oregon, 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

Lunch

Afternoon

- **Mahmoud A. Pouladi , UBC, Canada and Singapore**
 - TALENs to make new HD NHP Knock in Models
- **John R. Swart, Exemplar Genetics, USA**
 - Homologous recombination and somatic cell nuclear transfer to generate new Knock in HD minipig models
- **Paul Verma, South Australia (SARDI/Monash)**
 - Genome editing with TALENs and iPSC to engineer Large Animal Disease Models

Discussion Forum

Discussion Leaders: TBD

Dinner

Session III. Poster Viewing with Coffee and Desserts or Drinks

Tuesday, November 19

Breakfast

Morning

Session IV. Translatable Measures: Patients to Large Animal Models and Back

Chair: TBD

- **Jenny Morton, Cambridge, UK**
 - HD Sheep model
- **Nick Perentos, Cambridge UK**
- In vivo Electroencephalography and deep brain electrophysiology in sheep models of neurodegeneration

Coffee Break

- **Ralf Reilmann, Muenster, Germany**
 - TRACK-HD minipig
- **Anthony Chan, Emory Univ., USA**
 - HD Transgenic NHP for drug discovery

Discussion Forum

Discussion Leaders: TBD

Lunch

Session V. Histopathology and Molecular Phenotyping in Large Animal Models

Chair: TBD

- **Jim Gusella, MGH, Boston**
 - Perspectives from a Genetic Disease

Data Blitz:

- **Hana Hansikova, Prague, Czech Republic**
 - Mitochondrion, a source of energy and also a source of oxygen radicals
- **Stefan Juhas, Libechov, Czech Republic**
 - Immunohistochemistry of F2 generation of minipigs
- **Petr Solc, Libechov, Czech Republic**

- DNA damage
- **Suzanne Reid, University of Auckland, NZ**
 - Molecular and Histological Phenotypes in HD Sheep
- **Chiara Zuccato, Milano, Italy**
 - Exploring BDNF and cholesterol levels in the HD minipig

Coffee Break

Session VI. Invasive and Interventional Strategies using Large Animal Models

Chair: TBD

- **Carsten Reidies Bjarkam, Aarhus, DK**
 - The use of stereotaxic procedures in pigs, promises and pitfalls
- **Matt Gunis, Worcester MA**
 - Finding the striatum using MRI guided CT in the sheep
- **Neil Aronin, Worcester, USA**
 - Preclinical testing of AAV mediated Htt lowering therapies in HD sheep : Trials and Tribulations
- **Simon Bawden (SARDI)**
 - Challenges and Solutions for using Large Animal Models in Therapeutic Testing

Discussion Forum

Discussion leaders: TBD

**Closing Remarks: Jan Motlik, Libechov, Czech Republic
David Howland, CHDI, USA**

Dinner

Wednesday, November 20

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

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