



The 4th Animal Models of Neurodegenerative Diseases

Tentative Conference Program

October 2017, 22-24, Chateau Liblice, Czech Republic; www.amnd.cz

Five sessions focus on:

- 1. Perspectives from Cellular and Rodent Models for Neurodegenerative Diseases.
- 2. Progress in Technologies and Development of Large Animal Models of HD and ALS.
- 3. Perspectives of Gene Therapy in Neurodegenerative Diseases.
- 4. New DNA Damage and Mitochondria Biomarkers in Development of HD and ALS.
- 5. Development of New Cognitive and Behavioural Tests in Large Animal Models of HD.

Speakers include:

San Diago, USA Gene W. Yeo, Melvin Evers, Amsterdam, NL Edith L. Pfister, **Boston, USA** Munich, DE Nik Klymiuk, Carsten Bjarkam, Alborg, DK Bas Blits, Amsterdam, NL Slaven Erzeg, Valecia, ES William Singleton, **Bristol, UK** Libechov, CZ Zdenka Ellederova, Lars Eide, Oslo, NO Hana Hansikova, Prague, CZ **Huu Phuc Nguyen,** Tuebingen, DE Prague, CZ Jiri Klempir, Marian Hruska-Plochan, Zurich, CH Martin Marsala, San Diego, USA Ralf Reilmann, Muenster, DE

Petr Vodicka, Libechov, CR, Boston, USA

Conference is accompanied by presentations of leading companies in molecular and cell biology.

Sunday, October 22, 2017

Arriva

Complimentary shuttle from Prague Airport to Chateau Liblice is provided on demand.

Registration, Welcome Drink

Monday, October 23, 2017

Keynote Lecture:

Petr Svoboda, Prague, Czech Republic - A sobering look at the mammalian RNAi pathway.

Session 1: Perspectives from Cellular and Rodent Models for Neurodegenerative Diseases

Slaven Erzeg, Valencia, Spain - Induced pluripotent stem cells for modelling and treatment of human neurodegenerative diseases.

Marian Hruska-Plochan, Zurich, Switzerland - Interaction of DPRs alter their subcellular localization, aggregation pattern (and toxicity).

Huu Phuc Nhuyen, Tuebingen, Germany - Effects of lowering huntingtin in the Hypothalamus in the BACHD rat.

Petr Vodicka, Boston, USA / Libechov, Czech Republic – Stimulating autophagy and reducing inclusions in mouse model of Huntington's disease via expression of transcription factor EB (TFEB).

Helena Skalnikova, Libechov, Czech Republic - Exosomes as potential carriers of biomarkers in neurodegenerative diseases.

Marta Biagioli, Trento, Italy - Aberrant Alternative Splicing as a new player in Huntington's Disease pathogenesis.

Martin Balaštík, Prague, Czech Republic - Isomerase Pin1 as a regulator of stability and solubility of CRMP2 in aging and AD.

HD and ALS – Clinical view:

Jiri Klempir, Prague, Czech Republic – Living with neurodegeneration

Session 2: Progress in Technologies and Development of Large Animal Models of HD and ALS

Carsten Bjarkam Alborg, DK - Cytoarchitecture and surface anatomy of the Göttingen minipig telencephalon.

William Singleton, Bristol, UK - Functional and translational performance of a novel recessed-step catheter and stereotactic system for use in pre-clinical and clinical CED indications

Bas Blits, Amsterdam, NL - AAV mediated delivery in large animals.

Martin Marsala, San Diego, USA - Potent anti-spasticity and anti-nociceptive effect of subpial GAD65 and VGAT delivery in rat and mice.

Video conference

Gene W. Yeo, San Diego, USA - RNA targeting CRISPR technologies for disease applications

Poster Session

Evening program

Concert and dance Bratislava Hot Serenaders

www.serenaders.sk

Tuesday, October 24, 2017

Session 3: Perspectives of Gene Therapy in Neurodegenerative Diseases

Nik Klymiuk, Munich, Germany - Gene Editing for Tailoring Pig Models for Monogenic Diseases.

Melvin Evers, Amsterdam, NL - Establishing proof-of-concept of gene therapy for Huntington's disease.

Edith L. Pfister, Boston, USA – Reduction of Huntingtin by an scAAV9 artificial miRNA in large animals.

Petra Vochozkova, Munich, Germany / Libechov, Czech Republic - Evaluation of strategies for humanization of the entire porcine HTT locus.

Session 4: New DNA Damage and Mitochondria Biomarkers in Development of HD and ALS

Lars Eide, Oslo, Norway - New DNA Damage and Mitochondria Biomarkers in Development of HD and ALS.

Hana Hansikova, Prague, Czech Republic - Mitochondrial phenotype in minipig model transgenic for N-terminal part of human mutated huntingtin.

Petra Šmatlíková, Libechov, Czech Republic - Oxidative stress in Primary Porcine Fibroblasts Expressing Mutated Huntingtin.

Petr Šolc, **Libechov**, **Czech Republic** - DNA damage response in the development of Huntington's disease.

Michaela Vaškovičová, Libechov, Czech Republic - Double strand DNA breaks response in Huntington's disease transgenic minipigs.

Session 5: Development of New Cognitive and Behavioural Tests in Large Animal Models of HD Zdenka Ellederova, Libechov, Czech Republic - Following the phenotype development of

Zdenka Ellederova, Libechov, Czech Republic - Following the phenotype development of TgHD minipigs by invasive and noninvasive approach.

Verena Schuldenzucker, Muenster, Germany - Longitudinal phenotyping of tgHD minipigs.

Ralf Reilmann, Muenster, Germany - The minipig model in Huntington Disease - opportunities and challenges.

Closing of the Conference

Wednesday, October 25, 2017

Morning excursion - the PIGMOD Center minipig facility, Pavilion of Surgery Unit and Pavilion of Biomedicine in Libechov.