



Final WORKSHOP of the BRAINVECTORS European Consortium.

## NEUROTROPHIC FACTOR GENE DELIVERY FOR NEURODEGENERATIVE DISEASES: STILL A PROMISING CLINICAL PARADIGM?

12-14 September 2016 - Auditorium Alexandre Yersin, Lausanne University Hospital, CHUV, rue du Bugnon 48, Lausanne, Switzerland

### PROGRAM

#### September 12th

12:00-13:00 Registration and posters hanging.

12:00-13:30 *Lunch*

13:45 -14:00 Welcome addresses:

**Liliane Tenenbaum**, Lausanne University Hospital, BrainVectors coordinator

**Nicole Déglon**, Head of the Center for Neuroscience Research, Lausanne University Hospital.

**Renaud Du Pasquier**, Head of the Neurology Service, Lausanne University Hospital.

**Session 1: Neuroprotective gene therapy for neurodegenerative diseases** Chairman: Marc Levivier

14:00-14:45 **Mart Saarma**, Helsinki (FI), *Potential of neurotrophic factors for Parkinson's disease*

14:45-15:30 **Krys Bankiewicz**, San Francisco (CA-USA), *AAV-mediated neurotrophic factor gene delivery : updates of clinical trials for Parkinson's and Alzheimer diseases.*

*Coffee break*

15h45 - 16:30 **Stéphane Palfi**, Paris (FR), *The place of gene therapy for Parkinson's disease among existing treatments: history of the ProSavin clinical trial .*

16:30 - 17:00 **Pavlina Konstantinova**, Amsterdam (NL), *Public acceptance of Gene therapy products: risk:benefit ratio.*

17:00-18:30 **Round table: The future of neurotrophic gene therapy for Parkinson's disease.**

**Mart Saarma**, Helsinki (FI), **Krystof Bankiewicz**, San Francisco (CA-USA), **Harald Petry**, Amsterdam (NL), **Stéphane Palfi**, Paris (FR), **José-Luis Lanciego**, Pamplona (SP).

Moderator: **Liliane Tenenbaum**.

**September 13th**

**Session 2: Optimization of neurotrophic factor gene delivery** Chairmen: Mart Saarma and Sebastian Kügler

9:00-9:30 **Tomas Gonzales-Hernandez**, Tenerife (SP) *Dose- and time-dependent effects of GDNF on the rat mesostriatal system: a rationale for controlling GDNF release.*

9:30 -10:00 **Sebastian Kügler**, Göttingen (D) *Discontinuous GDNF delivery by GeneSwitch vectors*

10:00-10:30 **Mikko Airavaara**, Helsinki (FI) *Will new neurotrophic factors reveal the same limitations? The example of CDNF.*

*Coffee break*

**Session 3: The BrainVectors industry-academia joint program** Chairmen: Eric Kremer and Otto Merten

11:00- 11:15 **Liliane Tenenbaum**, Lausanne (CH) *Presentation of the BrainVectors consortium*

11:15-11:45 **Eric Kremer**, Montpellier (F) *Why a dog virus could be a neurobiologist's best friend?*

11:45 -12:15 **Cecilia Lundberg**, Lund (S) *Post-transcriptional regulation of transgene expression*

12:15-12:45 **Atze Das**, Amsterdam (NL) *Tet systems for doxycycline-controlled gene expression*

13:00-14:30 *Lunch*

*Short communication by BrainVectors fellows*

14:30 -14:45 **Marie Humbert-Claude**, Lausanne (CH) *Pharmacological control of GDNF biological effects in the brain by clinically-acceptable dox doses using a sensitive inducible AAV vector.*

14:45 -15:00 **Diego Pignataro**, Pamplona, (SP) *The development of new molecular tools for AAV-mediated CNS transduction.*

15:00-15:15: **Shelby Shrigley**, Lund (SE) *Inducible and cell-specific expression of GDNF using lentiviral vectors.*

15:30-17:00 **Poster session** + *coffee*

18:30 – 22:00 *Social event (restricted to invited speakers).*

**September 14th**

**Session 4: *Preclinical evaluation of cellular and molecular therapies in the CNS*** . Chairperson: Liliane Tenenbaum

9:30- 10:00 **Jose-Luis Lanciego**, Pamplona (SP) *Gene therapy approaches for the treatment of Gaucher-related synucleinopathies like Parkinson's disease and dementia with Lewy bodies.*

10:00-10:30 **Jocelyne Bloch**, Lausanne (CH) *Preclinical evaluation of adult brain cell autotransplantation in the MPTP non-human primate model.*

*Coffee break*

11:00-11:30 **Nicole Déglon**, Lausanne (CH) *Molecular therapies for Huntington's disease.*

11:30-12:00 **Bernard Schneider**, Lausanne (CH) *Gene therapy for amyotrophic lateral sclerosis: the importance of non-cell autonomous disease mechanisms.*

12:00-12:30 **Nicolas Serratrice**, Lausanne (CH) *MPS VII Gene therapy : From the bench to clinic with CAV-2 vectors.*

12:30-12:40 **Nicole Déglon**, Lausanne (CH) *Presentation of the Swiss Transmed gene therapy network.*

*Lunch 12:45-14:00*

**Session 5: *Gene therapy clinical trials setup and monitoring*** Chairmen: **Hueseyin Firat** and **Otto Merten**

14:00-14:30 **Ana Filipa Rodrigues**, Oeiras (PT) *Impact of the cell source on the production of clinical-grade vectors: cGMP considerations and cell line engineering perspectives.*

14:30-15:00 **Salih Moussaoui**, Huningue (F) *Peripheral biomarkers of dementia of Alzheimer and Parkinson types.*

15:00 - 15:30 **Marie-Lise Gougeon** , Paris (F) *Immune response after rAAV2/5-hNAGLU injection in the brain of children with Sanfilippo type B syndrome.*

15:30-16:45. **Round table: *Final conclusions and future directions of brain gene therapy: expectations and limitation issues***

**Krystof Bankiewicz**, **Hueseyin Firat**, **Nicole Déglon**, **Marie-Lise Gougeon** and **Marc Levivier**.  
Moderator: **Pavlina Konstantinova**.

**End of BrainVectors workshop**

\*\*\*\*\*

All participants are kindly invited to attend the seminar of **Krystof Bankiewicz**, at the Auditorium **Mathias Mayor** in the "Département des Neurosciences Cliniques" (same building) in the frame of the:

**Colloque du Département des Neurosciences Cliniques / Seminars of the Clinical Neurosciences Department.**

Chairman: **Marc Levivier**.

17:00-18:00 **Krystof Bankiewicz**, San Francisco (CA-USA) *Image-guided vector delivery to the putamen with AAV2-AADC vector in Parkinson's disease and AADC deficiency.*