September 12th

11:00 - 12:30  Registration + posters hanging

12:30 - 13:45  Lunch

13:45 - 14:00  Welcome addresses:
Dr Liliane Tenenbaum, BrainVectors project coordinator
Prof. Philippe Ryvlin, head of the Clinical Neuroscience Department.

14:00 - 18:00  Session I: Neuroprotective gene therapy for Parkinson’s disease

14:00 - 14:30  Mart Saarma, Helsinki, Finland
Potential of neurotrophic factors for Parkinson’s disease

14:30 - 15:00  Raymond Bartus, San Diego, USA-CA (To be confirmed)
Lessons from the AAV2-NTN Clinical trials

15:00 - 15:30  Coffee break

15:30 - 16:00  Krys Bankiewicz, San Diego, USA-CA
Update on the AAV2 GDNF Parkinson’s disease clinical trial
16:00 - 16:30  **Pavlina Konstantinova**, UniQure, The Netherlands  
*Public acceptance of Gene therapy products: (risk:benefit ratio)*

16:30 - 17:00  **Christian Wider**, CHUV, Lausanne, Switzerland  
*Gene therapy for PD, an option among existing treatments?*

17:00 - 18:00  **Round table**: The future of neurotrophic gene therapy for Parkinson’s disease.  
**Mart Saarma**, **Krystof Bankiewicz**, **Raymond Bartus**, **Christian Wider**  
**Moderator**: **Liliane Tenenbaum**

20:00  Dinner

**September 13**th

9:00 - 12:30  **Session II**: Optimization of neurotrophic factor gene delivery

9:00 - 9:30  **Tomas Gonzales-Hernandez**, Spain  
*Dose-dependent neurochemical effects of GDNF in the rat brain: a rationale for adjusting the dose.*

9:30 - 10:00  **Sebastian Kügler**, Göttingen, Germany  
*Modalities of GDNF delivery: continuous versus pulses*

10:00 - 10:30  **Eric Kremer**, CNRS, Montpellier, France  
*Canine adenovirus-based vectors: retrograde transport, an advantage for GDNF delivery?*

10:30 - 11:00  Coffee break

11:00 - 11:30  **Mikko Airavaara**, Helsinki, Finland.  
*Will new neurotrophic factors reveal the same limitations? The example of CDNF*

11:30 - 12:00  **Brandon Harvey**, NIH, Bethesda, USA-ML (To be confirmed)  
*Regulating therapeutic NTF delivery based on disease state*

12:00 - 12:30  **Jocelyne Bloch**, Lausanne, Switzerland  
*Preclinical evaluation of cellular and molecular therapies in the MPTP non-human primate Model*

12:30 – 14:00  Lunch

14:00 - 15:30  **Session III**: Safety I: Regulation of transgene expression.

14:00 – 14:15  **Liliane Tenenbaum**, Lausanne, Switzerland  
*Presentation of the BrainVectors project*

14:15 - 14:45  **Atse Das**, Amsterdam, The Netherlands  
*Transcriptional regulation: the tet system*
14:45 - 15:15  Cecilia Lundberg, Lund, Sweden  
Post-transcriptional regulation of transgene expression

15:15 – 15:45  Gloria Gonzalez-Aseguinolaza Pamplona, Spain  
Cell-type specific inducible transgene expression in the brain

15:45 – 16:15  Coffee break

16:15 - 17:30  Session IV:  Safety II: immune responses to vectors and transgenes

16:15 – 16:45  Marc Tardieu, Paris, France  
Immune response to AAV capsids and transgenes injected in the brain

16:45 – 17:15  Isabella Saggio, Rome, Italy (to be confirmed)  
Vectors - cells interactions

17:15 - 18:30  Poster session (with cocktail)

**September 14th**

9:00 - 12:30  Session V:  Gene therapy clinical trials setup and monitoring

9:00 - 9:30  Paula Alves, Oeiras, Portugal  
Production of clinical-grade vectors: Technological innovations and cGMP rules for biological products

9:30 - 10:00  Salïha Moussaoui, Huningue, France  
Immunological detection of biomarkers in the blood of Alzheimer disease patients

10:00 - 10:20  Coffee break

10:20 - 11:30  Short communications by BrainVectors fellows

10:20 - 10:30  Abdelwahed Chtarto, Brussels University  
AAV-tetON vectors

10:30 - 10:40  Ludivine Breger, Lund, Sweden  
LV-tetON vectors

10:40 - 10:50  Felix Junyent, Montpellier, France  
Cav2-tetON vectors

10:50 – 11:00  Marie Humbert-Claude, Lausanne, Switzerland  
Pharmacological control of GDNF biological effects in the brain by clinically-acceptable dox doses using a sensitive inducible AAV vector

11:00 - 11:10  François Legueux, Huningue, France  
Inducible vectors: more safety issues? Immune response to the tet-transactivator
11:10 - 11:30  2 selected presentations by young scientists.

11:30 - 12:30  **Round Table:**  *Is it necessary to regulate neurotrophic factor gene delivery?*
Sebastian Kügler, Harald Petry, Mart Saarma, Tomas Gonzalez-Hernandez
*Moderator:* Cecilia Lundberg

12:30 – 14:00  Lunch

14:00-16:00  **Session VI:**  *Gene therapy for neurological diseases based on correction of genetic deficiencies.*

14:00 - 14:30  **Marc Tardieu,** Paris, France
*The San Filippo clinical trials. Feasibility of compensating genetic deficiencies in the child’s brain using AAV-mediated gene transfer*

14:30 - 15:00  **Nicole Deglon,** Lausanne, Switzerland
*Molecular therapies for Huntington’s disease.*

15:00 – 15:30  Coffee break

15:30 - 15:40  **Nicole Deglon**
*Presentation of the Swiss Transmed gene therapy network*

Krystof Bankiewicz, Raymond Bartus, Christian Wider, Harald Petry, Nicole Deglon
*Moderator:* Liliane Tenenbaum

**END OF THE WORKSHOP**