



# CliniGene

*2nd TRAINING COURSE*

*VIRAL AND NON-VIRAL VECTORS FOR  
GENE THERAPY APPROACHES TO HUMAN  
DISEASES: NEW DEVELOPMENTS*

## **Program**



**BARCELONA, January 22-23, 2009**  
**Veterinary School, Universitat Autònoma Barcelona**

## **Objective of the course**

The 2nd CLINIGENE Training Course will **aim at fostering the networking of the junior members of the consortium** by providing a forum for presenting and discussing the current status and the future of their research projects. This will occur in the context of dedicated sessions which also represent the main backbone of the meeting. In addition to providing scientific advice, this exercise is expected to improve personal relationships between the attendees and uncovering potential new areas of scientific collaboration. In addition to the oral presentations from the young investigators (selected from the submitted abstracts), the course will include 11 lectures from senior members of CLINIGENE that will update the last developments in specific vector technology and in gene therapy clinical trials. Furthermore, senior members of CLINIGENE will also serve as chairman/discussant of each session to supervise and promote discussions between participants. Two young investigators will also be involved as discussants in each session of project presentation.

In order to improve interaction between early stage researchers, two young investigators from every CLINIGENE Partner's laboratory should participate and send abstracts. All abstract will be presented as posters (to facilitate further discussions), although about 30 of them will also be selected for oral presentation. Lunch and coffee breaks will be served at the poster area.

### ***Directors of the course:***

**Fatima Bosch**

**Odile Cohen-Haguenuer**

### ***Scientific secretariat:***

**Sabrina Tafuro**

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### ***Organizing secretariat:***

**Celia Tunc**

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**Dead lines:**

Registration dead line: December 20<sup>th</sup> 2008

Abstract submission dead line: January 5<sup>th</sup> 2009

**Participation Fees:**

- Travel and subsistence costs of the speakers will be covered by CLINIGENE.
- The organization will also cover lunches (on 22<sup>nd</sup> & 23<sup>rd</sup>), dinners (on 21<sup>st</sup> & 22<sup>nd</sup>) and two nights accommodation (Wednesday Jan. 21<sup>st</sup> & Thursday Jan. 22<sup>nd</sup>) for all participants.
- Each partner will have to cover for the travel of the young members of their labs. Five fellowships of 150 euros each will be allocated to the authors of the best abstracts presented.

## Thursday, January 22

8:30-8:50 Registration open

8:50-9:00 **Welcome address and introductory remarks.**  
*Fatima Bosch* (CBATEG - Universitat Autònoma Barcelona)

### Session I: Non viral vectors

**Chairman:** *Nicole Deglon*

9:00-9:45 **Lecture 1**  
**Physical and Chemical methods for in vitro and in vivo gene transfer**  
*Lluís Mir* (CNRS-Institut Gustave-Roussy, Paris)

9:45-10:30 **Lecture 2**  
**Non viral vectors-from plasmid to minicircle DNA.**  
*Martin Schleef* (Plasmid Factory GmbH & Co. KG, Bielefeld)

10:30-11:00 **Coffee Break**

11:00-12:00 **Project presentation I: Non Viral Vectors**

**Chairman:** *Nicole Deglon*

**Discussants:** *Xavier M. Anguela, Karine Cambon*

**A new method to apply nanosecond pulsed electric field in vitro and in vivo**

*Julien Villemejeane* (CNRS-Institut Gustave-Roussy, Paris)

**Electrical characterization of new devices for in vitro nanopulse application**

*Aude Silve* (CNRS-Institut Gustave-Roussy, Paris)

**Immunotoxicology and tissue response profiles of electro gene transfer (EGT) in skeletal muscle by microarray analysis**

*Christopher Mann* (CBATEG - Universitat Autònoma Barcelona)

**A novel non-viral and viral technology for ex-vivo gene therapy: Magselectofection**

*Yolanda Sanchez Antequera* (Institute of experimental oncology, TUM, Munich)

**Session II: Retro- and Lentiviral vectors**

**Chairman:** *Lluis Mir*

**12:00-12:45 Lecture 3**

**Dissecting neurological function through gene transfer**

*Nicole Deglon* (CEA, Institut d'Imagerie Biomédicale (I<sup>2</sup>BM), France)

**12:45- 13:30 Project presentation II: Retro- and Lentiviral vectors**

**Chairman:** *Lluis Mir*

**Discussants:** *Marie Breton, Efren Riu*

**RNA interference as therapeutic strategy to counteract Huntington's disease**

*Karine Cambon* (CEA, Institut d'Imagerie Biomédicale (I<sup>2</sup>BM), France)

**Synthetic expression systems for the modulation of endogenous cellular networks**

*Stephanie Sievers* (RDIF/MSYS, Helmholtz)

**Exploitation of defined chromosomal loci for Retro- and Lentiviral vector production**

*Leonor Gama-Norton* (RDIF/MSYS, Helmholtz)

**13:30-14:45 Poster Presentation & Lunch**

**14:45- 15:30 Lecture 4**

**Use of retrovirus-based virus-like particles as vaccines**

*David Klatzmann* (Laboratoire de Biologie et Thérapeutique des Pathologies Immunitaires, APHP, Paris)

**15:30-16:30 Project presentation II: Retro- and Lentiviral vectors (cont.)**

**Chairman:** *Lluis Mir*

**Discussants:** *Christian Bressy, Efren Riu*

**Evaluation of transcriptionnally-regulated novel oncolytic viruses**

*Gaunghua Yang* (ENSC, Cachan)

**Human natural regulatory T-cells encoding the thymidine kinase suicide gene as a treatment for Inclusion Body Myositis patients**

*Yves Allenbach* (APHP, Paris)

**Improved lentiviral vectors for intrathymic gene transfer application**

*Isabelle Ragon* (APHP, Paris)

**New strategies in downstream processing of enveloped viruses for gene therapy – the baculovirus case study**

*Tiago Vicente* (IBET, Lisboa)

**16:30-17:00**      **Coffee Break**

**17:00-17:45** **Lecture 5**

**Addressing integrating vectors safety & ethical considerations**

*Odile Cohen-Haguenaer* (Clinigene-NoE Coordinator & L.B.P.A., Ecole Normale Supérieure de Cachan)

**Session III: Adenoviral vectors**

***Chairman: Martin Schleef***

**17:45-18:30** **Lecture 6**

**Adenovirus biology and vector development**

*Stefan Kochanek* (University of Ulm-Division of Gene Therapy)

**18:45** **Bus leaves Veterinary School to the Hotel and then to Barcelona**

**20:30** **Dinner at “El Gran Café”**

# Friday, January 23

## Session IV: AAV vectors

**Chairman:** David Klatzmann

**8:30-9:15**    **Lecture 7**  
**Gene Therapy Approches for Diabetes**  
*Fatima Bosch* (CBATEG - Universitat Autònoma Barcelona)

**9:15-10:15**    **Projects presentation III: Adeno and AAV vectors**

**Chairman:** David Klatzmann

**Discussants:** Verónica Jiménez, Julien Villemejeane

**Optimizing AAV production and usage of Reference Standard Stocks**  
*Eduard Ayuso* (CBATEG - Universitat Autònoma Barcelona)

**Evaluation of the in situ rAAV genome epigenetic status after long term transgene expression by bisulfite pyrosequencing, in nonhuman primate**  
*Adrien Leger* (CHU, Nantes)

**Analyses of AAV concatemers and proviruses by massive parallel pyrosequencing**  
*Ali Nowrouzi* (NCT, Heidelberg)

**Analysis of AAV tropism to skin tissues**  
*Noemie Alvarez-Pereyre* (Instiute for Medical Research. Hebrew University of Jerusalem)

**Cloning of the canine rpgr1p1 cDNA, mutated in the Miniature Longhaired Dachshund affected by Leber congenital amaurosis.**  
*Elsa Lhériveau* (CHU, Nantes)

**10:30-10:45**            **Coffee Break**

**10:45-12:00**    **Projects presentation III: Adeno and AAV vectors (cont.)**

**AAV-mediated PEDF gene transfer to a transgenic mouse model of diabetic retinopathy**  
*Pilar Villacampa* (CBATEG - Universitat Autònoma Barcelona)

**Subretinal delivery of AAV2 results in minimal immune responses that allow repeat vector administration in immunocompetent mice**

*Susie Barker* (UCL Institute of Ophthalmology, London)

**An in vivo, AAV-based selection strategy for the identification of novel genes promoting cardiomyocyte protection and regeneration**

*Giulia Ruozi* (ICGEB, Trieste)

**AAV-mediated Sulfamidase expression in the liver prevents development of somatic alterations in MPS IIIA mice.**

*Albert Ruzo* (CBATEG - Universitat Autònoma Barcelona)

**Tumor virotherapy based on tropism-modified oncolytic adenovirus**

*Christian Bressy* (CNRS-Institut Gustave-Roussy, Paris)

**12:00-13:30 : Collective interaction between young members of Clinigene**

*Fatima Bosch*

**1. Presentation of the “International Journal Club”**

**2. “The Clinigene Young Investigator Club”**

**3. Discussion about collaborative research between young members of Clinigene**

*Eduard Ayuso, Christopher Mann, Xavier M. Anguela and Carles Roca*

**12:30-15:00**      ***ExeCom meeting (only ExeCom members)  
(in paralell)***

**13:45-15:00**      ***Poster Presentation & Lunch***

**Session VI: Gene therapy Clinical Trials**

***Chairman: Odile Cohen-Haguenuer***

**15:00-15:45 Lecture 9**

**Manipulating regulatory T cells for immunotherapy of cancer and autoimmune diseases**

*David Klatzmann* (Laboratoire de Biologie et Thérapeutique des Pathologies Immunitaires, APHP, Paris)

**15:45 –16:30 Lecture 10**

**Therapeutic angiogenesis in the myocardium - from preclinical studies to clinical trials**

***Markku Nurminen*** (A.I.Vitanen Institute, University of Kuopio, Finland)

**16:30-16:40 Concluding remarks**

***Fatima Bosch*** (CBATEG - Universitat Autònoma Barcelona)

**16:40-17:00 Coffee break**

**17:00-18:00 Closing lecture**

**Targeting Gene Transfer to Improve the Safety and Efficacy of Gene Therapy**

***Keynote Speaker: Luigi Naldini*** (San Raffaele Telethon Institute for Gene Therapy (HSR-TIGET). Milan – ITALY)