



Dear colleagues,

The possibility to transfer genes in target cells, to modify gene expression or to repair gene mutations is tremendously useful to understand biological or pathological processes. It is also creating an emerging direction in human medicine, called gene therapy. Conditions as diverse as cancer, genetic disorders or infections are potentially treatable with various forms of gene therapy.

**Lentiviral vectors** are remarkable tools for gene transfer and for therapy. More sophisticated than any other viral gene transfer vector before them, their complex genetic engineering exploits the properties of lentiviruses (HIV, SIV, EIAV, FIV) while defining new tropisms and minimizing the risk of pathogenicity. With the ability to gene-modify a wide variety of cells including quiescent cells, lentiviral vectors are efficient and non-toxic gene transfer tools. They have found many applications, notably in the hematopoietic and central nervous systems. Concerns due to the occurrence of leukemias in patients treated with retroviral vectors have prompted an unprecedented interest in the biology of retroviral integration and insertional mutagenesis. In that regard, lentiviral vectors are also remarkable by their genomic integration pattern and low genotoxicity. Lentiviral vectors have evolved significantly over the past 10 years and continue to do so. Besides integrative gene transfer, vaccination or RNA interference, lentiviral vectors have also been developed for non-integrative approaches including site-specific gene correction. Nowadays, lentiviral vectors have reached the clinic. Regulatory agencies must evaluate the unique risks associated with the use of such technology in humans. The large-scale GMP production of such vectors is challenging but processes have been developed. Several clinical trials are now ongoing with HIV-1-derived vectors in AIDS, neurodegenerative disease or anemias. Still at a phase I/II stage, lentiviral vectors are nevertheless attracting scrutiny from industrials given the compelling attraction of molecularly-based medicine.

The European Community project **CONCERT** is a Life Science and Health scientific project for a concerted evaluation of the safety of retroviral transgenesis. Among other tasks, the project supports the development of lentiviral vectors for gene therapy for inherited diseases. CONCERT is also commissioned to educate and to disseminate knowledge. **GENETHON**, a partner of CONCERT, is a non-for profit biotechnology in the orphan diseases and gene therapy sectors. To meet the demand for information about lentiviral vectors, GENETHON and CONCERT have taken the initiative to organize this advanced scientific course at the **EVRY UNIVERSITY**, with the help of several institutional and industrial sponsors.

We offer an **international scientific symposium** that will present didactic, state-of-the art and comprehensive information on lentiviral vectors. This symposium is targeted to an audience of scientists in the academic, industrial and regulatory sectors. In addition, we are offering a **practical laboratory session** to a limited group of motivated participants willing to learn, hands-on, about HIV-1-derived lentiviral vectors. The practical course is organized by GENETHON. Thanks to the generosity of many sponsors, the training will be largely subsidised, thus facilitating the participation of many European students.

The events will take place at the **University of EVRY-VAL d'ESSONNE**, conveniently located South of PARIS. The campus of EVRY, at large, has a strong history and focus on the genome. It hosts several research Institutes, the AFM (French Muscular Dystrophy Association), a biotechnology park and Genopole<sup>R</sup>.

So, if you are interested in **learning** about basic concepts, performance and **novel directions** in **lentiviral vectorology** including state-of-the art **clinical applications**; if you want **hands-on practice** on these vectors with experts in lentiviral development and production, **this course is for you!**

We are looking forward to welcoming you in Evry.

Anne Galy, on behalf of the:

• **Scientific committee:**

Chris BAUM  
Marina CAVAZZANA-CALVO  
Mary COLLINS  
Francois-Loïc COSSET  
Olivier DANOS  
Anne GALY  
Stefan KARLSSON  
Luigi NALDINI  
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• **Local organizing committee:**

Anne GALY  
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