

# 7TH STEM CELL CLONALITY AND GENOME STABILITY RETREAT

Immediately prior to the Collaborative Congress of the European and Spanish Societies of Gene and Cell Therapy.



**Thursday, October 24th**  
**15.00-20.00:**

## *Genomics and Genotoxicity Platforms*

**Frederic Bushman:** *Methods for Analyzing Integration Site Distributions and Results from Recent Trails*

**Eugenio Montini:** *The Ying and the Yang of Vector Insertional Mutagenesis*

**Ute Modlich:** *Genotoxicity in Preclinical Mouse Models*

**Manfred Schmidt:** *Gene Therapy Vector Persistence, Stability, and Integrome Analyses in the Age of Next Generation Sequencing*

## *Stem Cells and Progeny*

**Alessandro Aiuti:** *Lentiviral Vector HSC Gene Therapy for WAS*

**Elena Almarza:** *Preclinical Studies for the Gene Therapy of LAD-I*

**Christopher Baum:** *Spontaneous and Induced Clonal Dominance*

**David Williams:** *BCL11A Modulation in SCD*

**Manuel Grez:** *Improved Gene Transfer Into Repopulating HSCs by Targeting CD133+ Cells*

**Friday, October 25th**  
**8.30-13.50:**

## *Thymus, T Cells, and Immunodeficiency*

**Bobby Gaspar:** *Lentiviral Vector Mediated Gene Therapy for ADA-SCID*

**Adrian Thrasher:** *Update on SCID-X1 Gene Therapy*

**Hans-Peter Kiem:** *Hematopoietic Stem Cell Gene Therapy for HIV/AIDS*

**Hans Reimer Rodewald:** *Progenitor Deprivation and T Cell Acute Lymphoblastic Leukemia*

**Sebastian Newrzela:** *Homeostatic Niches - Control Mechanisms in Mature T-cell Leukemia/Lymphoma*

## *Somatic Cell Reprogramming and Genetic Engineering*

**Axel Schambach:** *Residual Lentiviral LTRs in iPSC: A Useful Tool for Clonotypic Identification and Recombinase-Mediated Cassette Exchange*

**J.C. Izpisua-Belmonte:** *Fanconi Anemia and iPSCs*

**Zoltan Ivics:** *Genome Engineering with Sleeping Beauty Transposons*

**Toni Cathomen:** *Gene Editing in iPSC Disease Models*

**Luigi Naldini:** *Engineering Hematopoiesis for Treating Genetic Disease and Cancer*

## CONCLUDING ADDRESS: **Alessandra Biffi**

“Lentiviral HSC Gene Therapy for the Treatment of Metachromatic Leukodystrophy”



**Co-organizers:** Christopher Baum, Juan Bueren, Adrian Thrasher, and David Williams

Sponsored by the  
**Transatlantic Gene  
Therapy Consortium**

**Hotel Novotel Madrid  
Campo de Las Naciones**

**Madrid, Spain**

**October 24-25, 2013**

For Registration, please visit:  
<http://fluidsurveys.com/s/SCCGSR2013/>

Please contact  
Natasha Rossi  
([natasha.rossi@childrens.harvard.edu](mailto:natasha.rossi@childrens.harvard.edu))  
with any questions.