

5th Stem Cell Clonality and Genotoxicity Retreat

Friday, November 20, 2009; Location: Old Town Hall (City Center)

Session 1 Clinical situation (chair Nathalie Cartier and David A. Williams)	2:30-3:40 PM
Gene therapy of severe combined immunodeficiency Adrian Thrasher, London, UK	2:30-2:45
Gene expression profile and chromatin conformation of target cell lead to a cell-specific integration distribution of retroviral vector in lymphocytes and hematopoietic stem cells from ADA-SCID patients Alessandro Aiuti, Milano, Italy	2:45-3:00
Vector silencing, insertional activation of <i>EV11</i> and the induction of genomic instability in a gene therapy trial for Chronic Granulomatous Disease Manuel Grez, Frankfurt, Germany	3:00-3:15
Hematopoietic stem cell gene therapy for X-linked adrenoleukodystrophy: polyclonal distribution of lentiviral vector Nathalie Cartier, Paris, France	3:15-3:30
Questions to session 1	3:30-3:40
Session 2 Murine models and their relation to clinical observations (chair Cynthia Dunbar and Eugenio Montini)	3:40-4:50 PM
Murine Afr-SCID models : their role in predicting gene therapy safety Marina Cavazzana-Calvo, Paris, France	3:40-3:55
Lymphomagenesis in SCID-X1 mice following lentiviral vector-mediated gene delivery: is replicative stress a factor? Samantha Ginn, Westmead, Australia	3:55-4:10
A self-inactivating lentiviral vector for SCID-X1 gene therapy that does not activate LMO2 expression in human T cells Brian Sorrentino, Memphis, USA	4:10-4:25
Comparative analysis of vector integrations in vector-induced tumors reveals distinct requirements for oncogenesis between <i>Cdkn2a</i> ^{-/-} hematopoietic stem cells and committed cell lineages Eugenio Montini, Milano, Italy	4:25-4:40
Questions to session 2	4:40-4:40
Coffee break	4:50-5:15 PM
Session 3 Large animal models (chair Boris Fehse and Christopher Baum)	5:15-5:50 PM
Update on approaches to understanding and decreasing genotoxicity related to integrating vectors Cynthia Dunbar, Bethesda, USA	5:15-5:30
Clonality after positive selection of gene-modified hematopoietic cells Hans-Peter Kiem, Seattle, USA	5:30-5:45
Questions to session 3	5:45-5:50
Session 4 Homeostatic mechanisms (chair Dorothee von Laer and Punam Malik)	5:50-7:00 PM
Insertional transformation and induced suicide of gene-modified cells Boris Fehse, Hamburg, Germany	5:50-6:05
Same vector, different outcomes: External cues in the development of clonal dominance Christopher Baum, Hannover, Germany	6:05-6:20
T cell receptor monoclonality of T lymphocytes predisposes to transformation Dorothee von Laer, Frankfurt, Germany	6:20-6:35
Individual clone dynamics and the effect of clonal heterogeneity-lessons from a mathematical model analysis Ingo Roeder, Leipzig, Germany	6:35-6:50
Questions to session 4	6:50-7:00
Speakers' Dinner: 7:30 until 9:30 PM, Old Town Hall	

Saturday, November 21, 2009; Location: Conference Center at Hannover Messe

Session 5 Globin gene therapy (chair Marina Cavazzana-Calvo and Hans-Peter Kiem)	8:30-9:05 AM
Gene therapy for sickle cell disease and β -thalassemia using lentiviral vectors to enhance fetal hemoglobin production Derek Persons, Memphis, USA	8:30-8:45
Chromatin insulators: The good and the bad Punam Malik, Cincinnati, USA	8:45-9:00
Questions to session 5	9:00-9:05
Session 6 Integromics and Clonality (chair Mavilio and Bushman)	9:05-10:00 AM
HIV DNA integration: mechanism and consequences Frederic Bushman, Philadelphia, USA	9:05-9:20
Efficient pharmacodynamics studies in clinical gene therapy Christof von Kalle, Heidelberg, Germany	9:20-9:35
Highly clustered MLV integrations define cell-specific transcriptional regulatory elements in human hematopoietic progenitors Fulvio Mavilio, Modena, Italy	9:35-9:50
Questions to session 6	9:50-10:00
Coffee break	10:00-10:30 AM
Session 7 Alternative approaches (chair Toni Cathomen and Luigi Naldini)	10:30-11:40 AM
Developing site-specific integration systems with minimal perturbation of endogenous transcription at the target site Luigi Naldini, Milano, Italy	10:30-10:45
Transposons as nonviral, integrating gene vector systems Zoltan Ivics, Berlin, Germany	10:45-11:00
Zinc-finger nucleases: finding the balance between activity and toxicity Toni Cathomen, Hannover, Germany	11:00-11:15
Mechanism of episomal maintenance Wolfgang Hammerschmidt, München, Germany	11:15-11:30
Questions to session 7	11:30-11:40
Session 8 Alternative approaches (cont.) & regulatory issues (chair Xiaobin Victor Lu and Odile Cohen-Hagenauer)	11:40 -12:50 AM
piggyBac transposition to induce pluripotent stem cells Andras Nagy, Toronto, Canada	11:40-11:55
Directed integration of new insulated lentiviral vectors to the heterochromatin towards safer gene transfer to stem cells Odile Cohen-Hagenauer, Paris, France	11:55-12:10
Current FDA/CBER regulatory recommendations and considerations of gammaretroviral or lentiviral vector-based gene therapy products Xiaobin Victor Lu, Rockville, USA	12:10-12:25
Regulatory aspects of gene transfer medicinal products Matthias Schweizer, Langen, Germany	12:25-12:40
Questions to session 8	12:40-12:50 PM
Concluding remarks	12:50-1:00 PM
Lunch (for registered participants)	1:00-2:00 PM