FINAL PROGRAM
IVth Conference on Stem Cell Gene Therapy
September 13 to September 17, 2007
Thessaloniki, Halkidiki, Greece

THURSDAY, SEPTEMBER 13, 2007
6:00 pm NO HOST COCKTAILS
7:00 pm WELCOME RECEPTION

FRIDAY, SEPTEMBER 14, 2007
7:30 am BREAKFAST
8:30 am WELCOME
8:40 am SESSION I: GENE TRANSFER SYSTEMS FOR STEM CELL GENE THERAPY: STATE-OF-THE-ART AND NOVEL DEVELOPMENTS
Chairperson: David Bodine, National Institutes of Health, Bethesda, USA
8:40 am Luigi Naldini, San Raffaele Scientific Institute, Milano, Italy
“Improving safety and efficacy of HSC gene transfer by lentiviral vectors”
9:10 am George Vassilopoulos, Institute for Biomedical Research of the Academy of Athens, Athens, Greece
“Stem Cell Gene Transfer with Foamy Virus Vectors”
9:35 am Dennis Hickstein, National Cancer Institute, Bethesda, USA
“Successful gene therapy of canine leukocyte adhesion deficiency using foamy viral vectors”
10:00 am Christof von Kalle, National Centre for Tumour Diseases, Heidelberg, Germany
“The clonal fate of gene corrected myelo- and lymphopoiesis”
10:30 am COFFEE BREAK
11:00 am SESSION II: GENE TRANSFER SYSTEMS FOR STEM CELL GENE THERAPY: INSERTIONAL MUTAGENESIS AND GENOTOXICITY
Chairperson: Luigi Naldini, San Raffaele Scientific Institute, Milano, Italy
11:00 am Christopher Baum, Cincinnati Children's Hospital Medical Center, Cincinnati, USA
“Retroviral transgenesis and oncogenesis”
11:30 am Cynthia Dunbar, National Heart, Lung, and Blood Institute, Bethesda, USA
“Genotoxicity in the Rhesus Macaque Model”
12:00 pm Fulvio Mavilio, San Raffaele Scientific Institute, Milano, Italy
“Interactions between retroviral vectors and the genome of CD34+ hematopoietic cells”
12:30 pm  David Emery, University of Washington, Seattle, USA
“Reducing Retroviral Vector Genotoxicity with Chromatin Insulators”

1:00 pm  Eugenio Montini, San Raffaele Scientific Institute, Milano, Italy
“Modeling the genotoxicity of viral vector integration in a tumor prone hematopoietic stem cell transplantation model”

1:20 pm  BREAK FOR LUNCH

2:30 pm  POSTER SESSION A: POSTERS 1-23

6:30 pm  DINNER

7:30 pm  SESSION III: STEM CELL BIOLOGY AND LEUKEMIA STEM CELLS
Chairperson: Keith Humphries, BC Cancer Agency, Vancouver, Canada
7:30 pm  Stanton Gerson, Case Western Reserve University, Cleveland, USA
“DNA repair and the aging of stem cells”
8:00 pm  Stefan Karlsson, Lund University, Lund, Sweden
“The role of Smad signaling in hematopoiesis”
8:30 pm  Thalia Papayannopoulou, University of Washington, Seattle, USA
“Homing of ex vivo incubated cells: can it be improved?”
9:00 pm  Dominique Bonnet, Cancer Research UK, London, UK
“Leukaemic stem cells: new targets for therapies”
9:30 pm  Dorothee von Laer, Georg-Speyer-Haus, Frankfurt a. M., Germany
“A comparative analysis of the leukaemic potential of mature T cells versus T cell precursors”

SATURDAY, SEPTEMBER 15, 2007

7:30 am  BREAKFAST

8:30 am  SESSION IV: HEMATOPOIETIC STEM CELL EXPANSION AND IN VIVO SELECTION
Chairperson: Stefan Karlsson, Lund University, Lund, Sweden
8:30 am  Hans-Peter Kiem, Fred Hutchinson Cancer Research Center, Seattle, USA
“In vivo selection of gene-modified stem cells in a large animal model”
9:00 am  Keith Humphries, BC Cancer Agency, Vancouver, Canada
“Self-renewal programs as a target for gene therapy purposes”

9:30 am  SESSION V: GENE TARGETING AND REPAIR STRATEGIES
Chairperson: Kenneth Cornetta, Indiana University, Indianapolis, USA
9:30 am  David Russell, University of Washington, Seattle, USA
“Gene targeting with AAV”
10:00 am  Philip Gregory, Sangamo BioSciences Inc., Richmond, USA
“Precision Editing of the Human Genome with Engineered Zinc Finger Proteins”
10:30  Andre Lieber, University of Washington, Seattle, USA
“Adenovirus vectors for targeted integration into human hematopoietic stem cells”
11:00 am COFFEE BREAK

11:30 am SESSION VI: IMMUNE DEFICIENCY SYNDROMES
Chairperson: Claudio Bordignon, San Raffaele Scientific Institute, Milano, Italy
11:30 am Alessandro Aiuti, San Raffaele Scientific Institute, Milano, Italy
“Hematopoietic stem cell gene therapy for ADA-SCID”
12:00 pm Adrian Thrasher, Great Ormond Street Hospital for Children NHS Trust & UCL Institute of Child Health, London, UK
“Successful reconstitution of immunity following gene therapy for SCID”
12:30 pm Marina Cavazzana-Calvo, INSERM U768 and Université Paris Descartes, Paris, France
“Gene therapy targeting hematopoietic stem cells: from the bench to the bedside”
1:00 pm Brian Sorrentino, St. Jude Children’s Research Hospital, Memphis, USA
“Factors Involved in Transformation in XSCID Gene Therapy”

1:30 pm BREAK FOR LUNCH

2:30 pm POSTER SESSION B: POSTERS 24-46

6:30 pm DINNER

7:30 pm SESSION VI: continues
Chairperson: Claudio Bordignon, San Raffaele Scientific Institute, Milano, Italy
7:30 pm Tadashi Ariga, Hokkaido University, Sapporo, Japan
“Hematopoietic stem cell gene therapy for ADA deficiency without cytoreductive conditioning”
8:00 pm Fabio Candotti, National Human Genome Research Institute, Bethesda, USA
“Gene Therapy for ADA-deficiency in the USA”
8:30 pm Maria Grazia Roncarolo, San Raffaele Scientific Institute, Milano, Italy
“Gene therapy for Wiskott-Aldrich syndrome”

9:00 pm SESSION VII: CHRONIC GRANULOMATOUS DISEASE
Chairperson: Alessandro Aiuti, San Raffaele Scientific Institute, Milano, Italy
9:00 pm Manuel Grez, Johann Wolfgang Goethe Universität Frankfurt AM Main, Frankfurt, Germany
“Phase I Gene Therapy Study for X-CGD: Results, Lessons and Perspectives”
9:30 pm Harry Malech, National Institute of Allergy and Infectious Diseases, Bethesda, USA
“Update on Gene Therapy for Chronic Granulomatous Disease”

SUNDAY, SEPTEMBER 16, 2007

7:30 am BREAKFAST

8:30 am SESSION VIII: THALASSEMIA AND SICKLE CELL DISEASE
Chairperson: George Stamatoyannopoulos, University of Washington, Seattle, USA

8:30 am  Philippe Leboulch, Harvard Medical School and Brigham and Women's Hospital, Boston, USA
“Globin gene transfer and in vivo cell expansion”

9:00 am  Evangelia Yannaki, George Papanicolau Hospital Gene and Cell Therapy Center, Thessaloniki, Greece
“A mobilization study in β-major thalassemia”

9:30 am  Michel Sadelain, Memorial Sloan-Kettering Cancer Center, New York, USA
“Transcriptional and post-transcriptional gene regulation in stem cell-based gene therapy”

10:00 am  Giuliana Ferrari, San Raffaele Scientific Institute, Milano, Italy
“Preclinical assessment of gene therapy for beta thalassemia”

10:30 am  COFFEE BREAK

11:00 am  SESSION VIII: continues
Chairperson: George Stamatoyannopoulos, University of Washington, Seattle, USA
11:00 am  David Bodine, National Human Genome Research Institute, Bethesda, USA
“Novel vectors for globin gene therapy using regulatory elements from non-globin genes”

11:30 am  Punam Malik, Cincinnati Children's Hospital Medical Center, Cincinnati, USA
“Gene Therapy for Hemoglobinopathies”

12:00 pm  Derek Persons, St. Jude Children’s Research Hospital, Memphis, USA
“Gamma-Globin Lentiviral Vector-Mediated Stem Cell Gene Therapy of Beta-Thalassemia and Sickle Cell Disease”

12:30 pm  SESSION IX: FANCONI ANEMIA
Chairperson: Maria Grazia Roncarolo, San Raffaele Scientific Institute, Milano, Italy

12:30 pm  Juan Bueren, Ciemat, Research Centre for Energy, Environment and Technology, Madrid, Spain
“Towards the Gene Therapy of Fanconi Anemia Patients with Lentiviral Vectors”

1:00 pm  David Williams, Cincinnati Children's Hospital Medical Center, Cincinnati, USA
“Too little, too late: the initial experience in FA gene therapy”

1:30 pm  BREAK FOR LUNCH

3:00 pm  SESSION X: PANEL DISCUSSION: WHAT IS THE OPTIMAL BM CONDITIONING FOR STEM CELL GENE THERAPY?
Chairperson: Athanassios Fassas, George Papanicolau Hospital Gene and Cell Therapy Center, Thessaloniki, Greece
Guido Lucarelli, Mediterranean Institute of Haematology, Roma, Italy
Maria Grazia Roncarolo, San Raffaele Scientific Institute, Milano, Italy
Athanassios Fassas, George Papanicolau Hospital Gene and Cell Therapy Center, Thessaloniki, Greece
Philippe Leboulch, Harvard Medical School and Brigham and Women's Hospital, Boston, USA

General Discussion

4:00 pm  SESSION XI: PAPERS SELECTED FROM THE POSTERS

6:30  DINNER

7:30 pm  SESSION XII: GENE THERAPY OF CANCER

Chairperson: David Williams, Cincinnati Children's Hospital Medical Center, Cincinnati, USA

7:30 pm  Claudio Bordignon, San Raffaele Scientific Institute, Milano, Italy
“T cell-based immunogene therapy of cancer”

8:00 pm  David Williams, Cincinnati Children's Hospital Medical Center, Cincinnati, USA
“Too much of a good thing? MGMT in chemoresistance and brain tumor therapy”

8:30 pm  Attilio Bondanza, San Raffaele Scientific Institute, Milano, Italy
“Requirements for retroviral targeting of a suicide gene to alloreactive memory stem T cells for adoptive immunotherapy of leukemia”

9:00 pm  Masafumi Onodera, University of Tsukuba, Tsukuba Science City, Japan
“Opposite effects of an integration site on expression of the transgene - Gene silencing or Leukemogenesis”

9:30 pm  C. Anthony Blau, University of Washington, Seattle, USA
“Erythropoietin Blockade for the Treatment of Cancer”

MONDAY, SEPTEMBER 17, 2007

7:30 am  BREAKFAST

CONFERENCE ENDS